Patient Engagement open forum Into Reality

How Can Patient Engagement Foster Access Through Improved Affordability?

September 24th from 15.00 to 17.00 CET

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

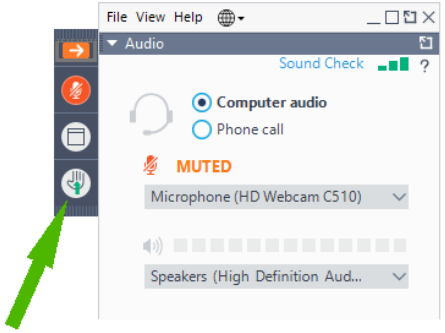
EUPATI
European Patients' Academy on Therapeutic Innovation

PATIENT FOCUSED MEDICINES DEVELOPMENT

Ground rules

- Please mute yourself during this webinar.
- If you want to ask a question, you can either ask through the Q&A box or by raising your hand.
- Our agenda is quite robust and we might need to select the questions to be answered to ensure all speakers have their statement.
- Enjoy!

Q&A



PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

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PATIENT FOCUSED MEDICINES DEVELOPMENT

Speakers

- **Tracy Swan**, International Treatment Preparedness Coalition
- **Diarmaid McDonald**, Just Treatment
- **Prof. Zoltán Kaló**, Center for Health Technology Assessment at Semmelweis University
- **Clare Hague**, Janssen
- **Dr Tamás Bereczky**, EUPATI Training Coordinator – moderator

Agenda

15.00 – 15.05 – Introduction to the PEOF and session by Tamás Bereczky

15.05 – 15.25 – Statements from panellists

- Tracy Swan–Patients working in policy matters –access and affordability
- Claire Hague –Access and affordability considerations in the pharmaceutical industry

15.25 – 15.45 – Q&A

15.45 – 16.05 – Statements from panellists

- Diarmaid McDonald-Patient organisations working in access and affordability
- Prof. Zoltán Kaló–What science knows about access and affordability

16.05 – 16.45 – Q&A and discussion

16.45 – 17.00 – Takeaway messages

Patient Engagement Open Forum (PEOF)

- Patient Engagement Open Forum is a series of virtual events (in 2020) where we will work together, in a multi-stakeholder context, **to turn patient engagement from an aspiration into reality**.

- The Forum aims to provide **a holistic perspective** of patient engagement, the **landscape and actors**, and **foster collaboration** and **co-creation** while **breaking down fragmentation** that are often present in patient engagement work.

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Patient Engagement Open Forum 2020 (PEOF) - link

June 25th

PEOF2020 opening plenary (PARADIGM, PFMD and EUPATI)

Parallel sessions:

- Patient Engagement tools session #1 (consultation organised by PARADIGM)
- Patient engagement within MedTech (panel organised by EUPATI)
- Patient experience in regulatory processes (workshop organised by PFMD)

June 26th

Parallel sessions:

- How to engage patients in the early phases? (workshop organised by PFMD)
- Patient engagement in co-creating plain language summaries (workshop organised by PFMD)
- National Health Council Patient Engagement Fair-Market Value Calculator Toolbox (organised by NHC)

July 9th

Parallel sessions:

- Patient Engagement tools session #2 (consultation organised by PARADIGM)
- Flash presentations
  1. Sustainability roadmap for the patient engagement ecosystem
  2. Patient engagement agreements explained
  3. Patient engagement in medicines R&D in the CEE region
- Motherhood should not be a fight – better safety information on medicines use during pregnancy and breastfeeding, with patients for patients. (Workshop organised by IMI-Conception)

September 10th

Plenary session

- PARADIGM Patient Engagement Toolbox (webinar organised by PARADIGM)
- Patient Engagement Monitoring and Evaluation Framework (workshop organised by PARADIGM)

September 24th

Parallel sessions:

- How PE can foster access through improved affordability? (webinar organised by EUPATI)
- Patient engagement in clinical trial phase or/and in the regulatory submission phase (workshop organised by PFMD – to be confirmed at a later date)
- From diagnosis to treatment and beyond: personalised medicine – what's in it for patients and how to make it available to patients who could benefit from it? (workshop supported by PFMD)

October 15th

Parallel sessions:

- Patient Engagement and Quality by Design: Co-Developing an Implementation Roadmap for Clinical Trials (organised by CTTI)
- Good Lay Summary Practice, communicating trial results to the general public – How patient engagement can work (organised by EFPIA and EFGCP)

November 5th

THEME: Regulatory

November 23rd

Plenary session:

- PEOF2020 conclusion session

How Patient Engagement Can Foster Access Through Improved Affordability?

Thursday 24 September 2020 from 15.00 to 17.00 CET

TRACY SWAN

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MEDICINES DEVELOPMENT

5g of diamonds

25 1-carat ($1900 each)

$48,000

5g of daclatasvir

12 weeks of treatment, 60mg/day

$63,000 (US price)

Slide courtesy of Dr Andrew Hill

HOW MUCH DOES IT COST TO DEVELOP A DRUG?

The pharmaceutical industry has not been transparent about the cost to bring a drug to market

A recent study estimated that cost ranges from $314 million to $2.8 billion, with a median of $985 million – including failed trials[1]

Another study included post-approval R&D costs; it estimated a cost of $2870 million per drug[2]

1. Woulters OJ, et al. Estimated Research and Development Investment Needed to Bring a New Medicine to Market, 2009-2018. JAMA . 2020 Mar 3;323(9):844-853. doi: 10.1001/jama.2020.1166.
2. De Masi JA, et al. Innovation in the pharmaceutical industry: New estimates of R&D costs
J Health Econ. 2016 May;47:20-33. doi: 10.1016/j.jhealeco.2016.01.012.

SOFOSBUVIR – a case study

HOW MUCH DID IT COST?

Gilead bought Pharmasset to get SOF, which was in phase II, for US $ 11 billion[1]

HOW MUCH DO PHASE III TRIALS COST ?

- The cost of a phase III trial is estimated at US $19 million[2]
- There were 1,945 people in Gilead's four phase III trials[3]

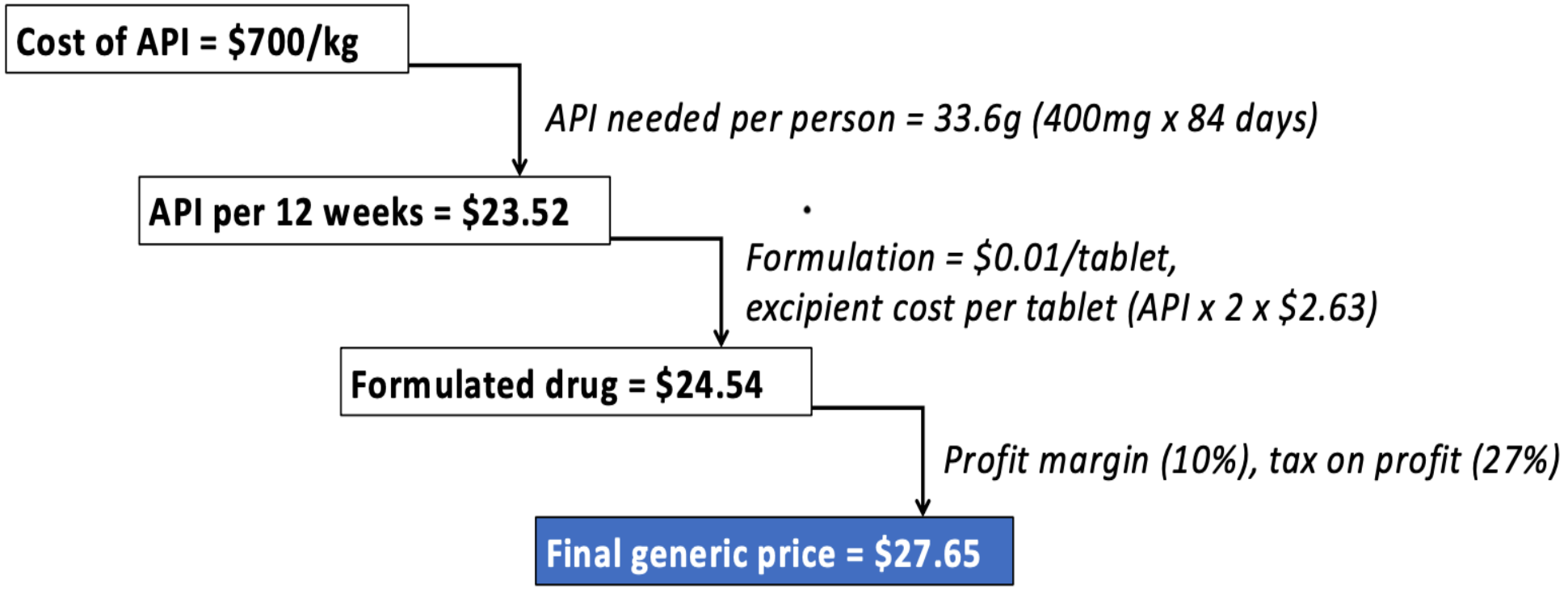
Safe to say US $ 200 million?

SOF revenue (2013- 2017) $ 31.5 billion

1. https://www.businesswire.com/news/home/20111121005623/en/Gilead-Sciences-Acquire-Pharmasset-11-Billion
2. https://www.jhsph.edu/news/news-releases/2018/cost-of-clinical-trials-for-new-drug-FDA-approval-are-fraction-of-total-tab.html
3. https://www.accessdata.fda.gov/drugsatfda_docs/label/2015/204671s002lbl.pdf

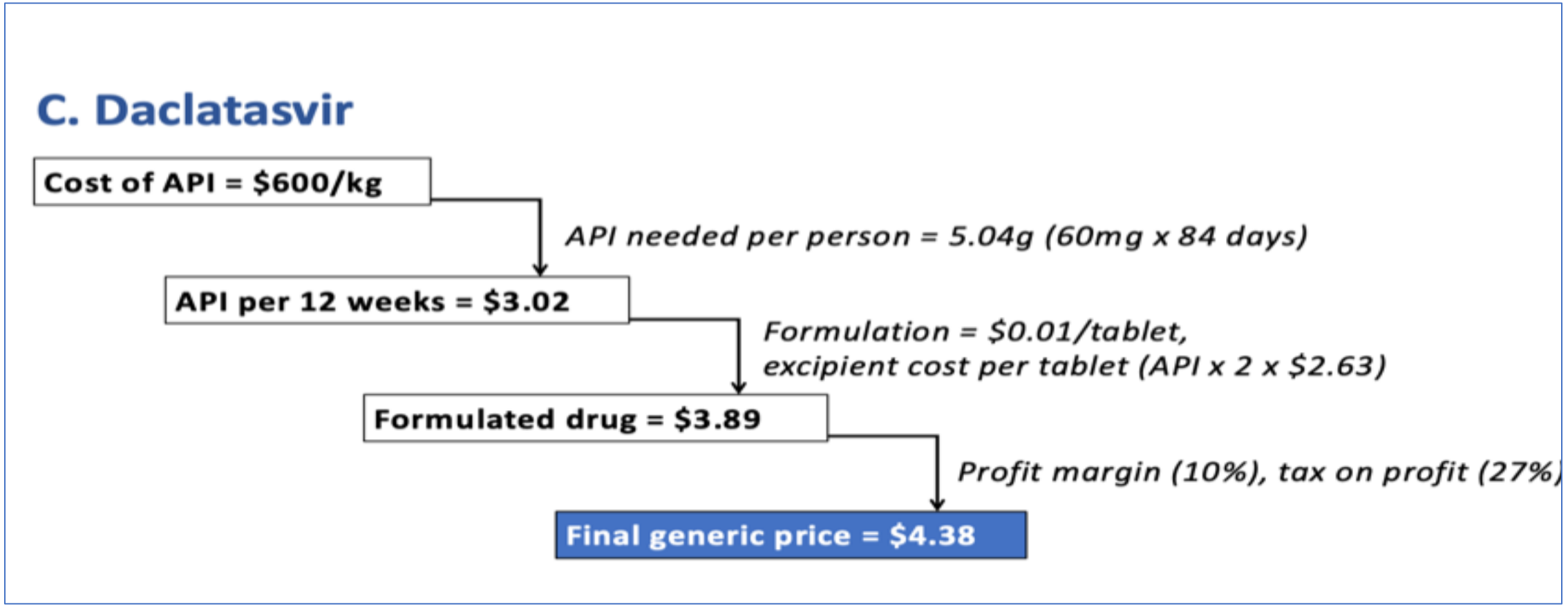
What does it cost to profitably mass-produce SOF?

Sofosbuvir



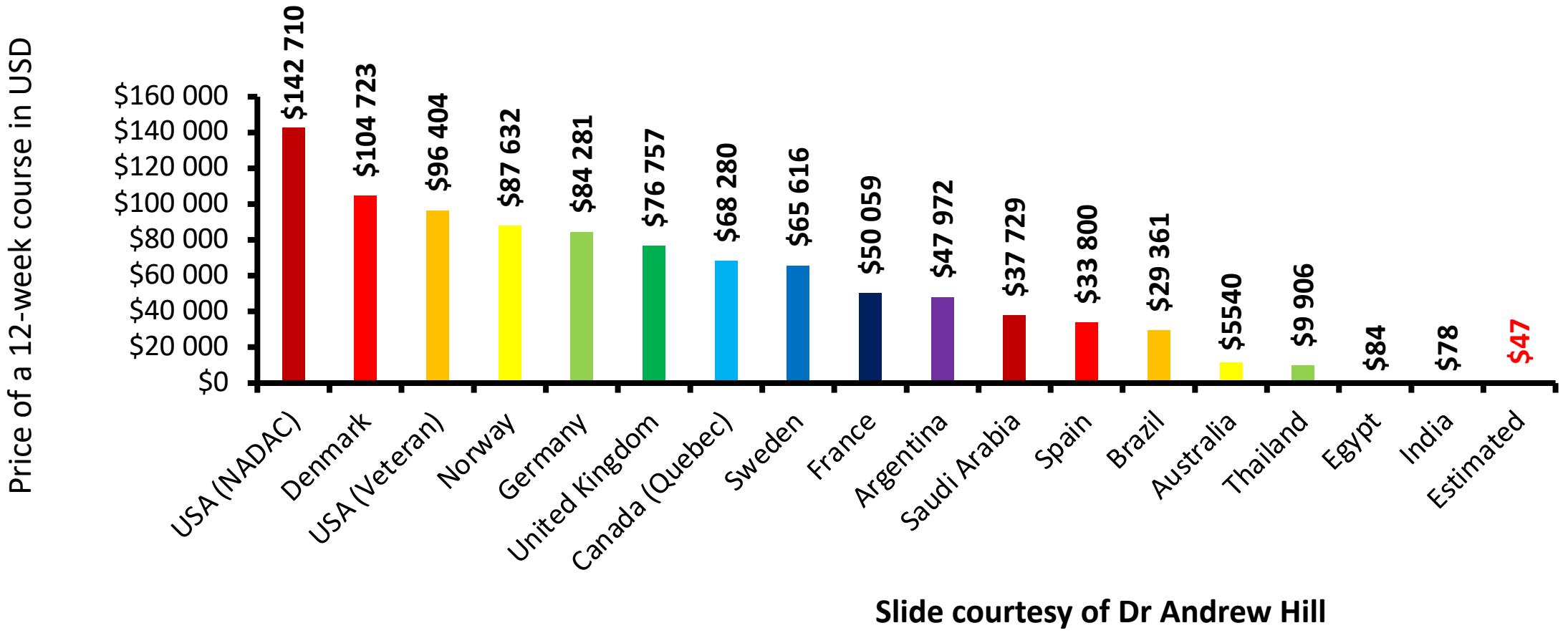
Slide courtesy of Dr Andrew Hill

What does it cost to profitably mass-produce DCV?



Slide courtesy of Dr Andrew Hill

Prices for 12 weeks of SOF/DCV by Country



Slide courtesy of Dr Andrew Hill

Patient Engagement open forum Into Reality

How Patient Engagement Can Foster Access Through Improved Affordability?

Access & Affordability Considerations in the Pharmaceutical Industry

Clare Hague, PhD. Janssen EMEA

Thursday 24 September 2020 from 15.00 to 17.00 CET

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PATIENT FOCUSED MEDICINES DEVELOPMENT

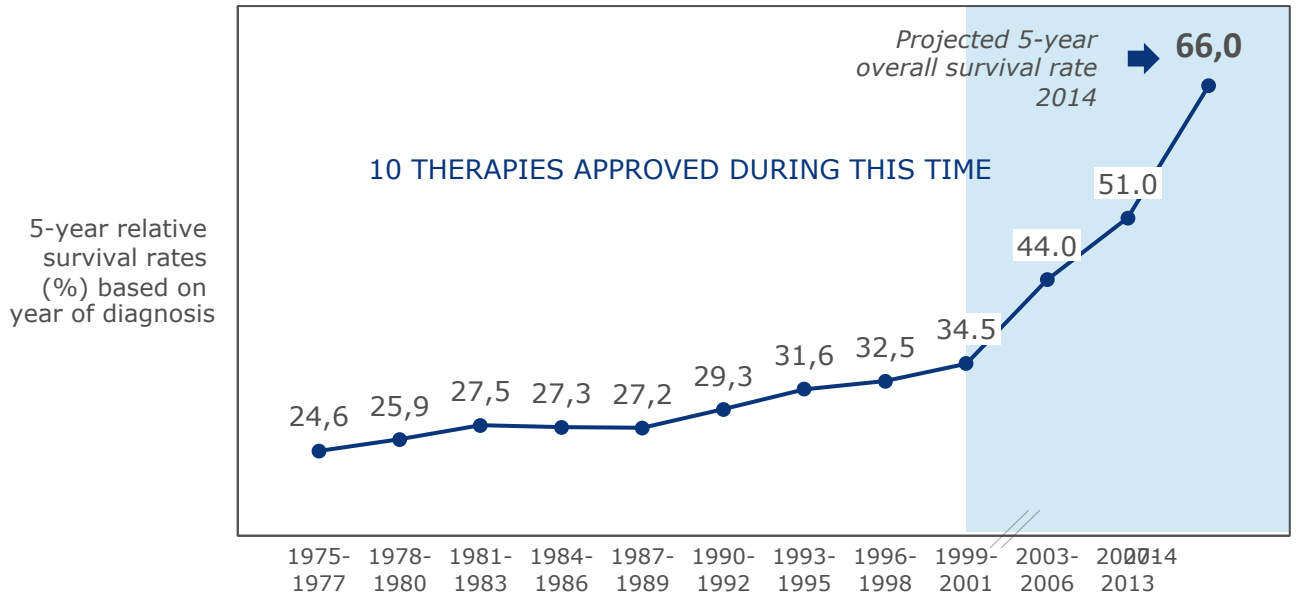
Today's Presentation

HOW WE NAVIGATE ACCESS CHALLENGES IN EUROPE

HOW CAN WE WORK TOGETHER TO DO MORE FOR PATIENTS

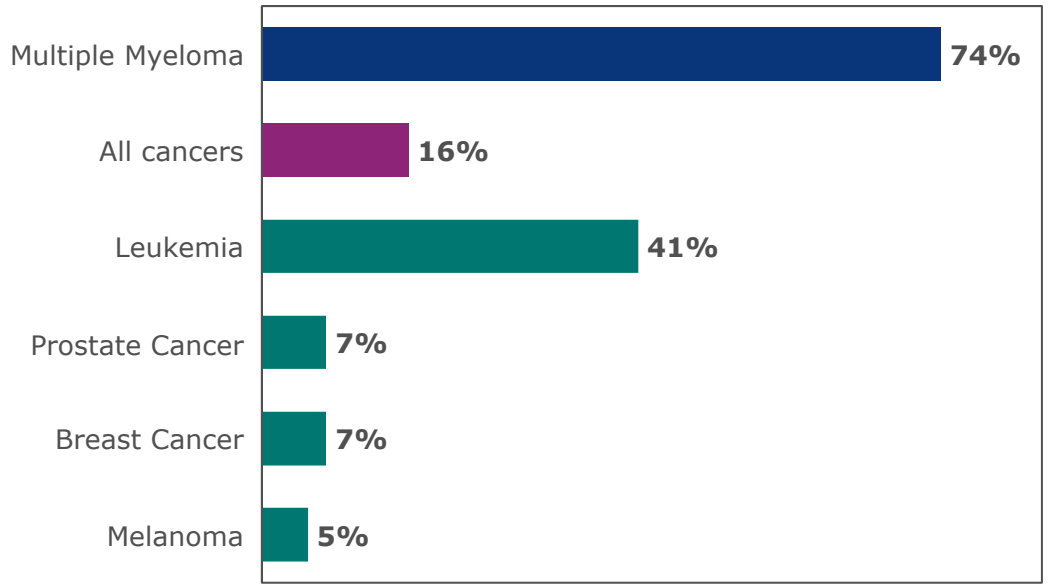
Innovative medicines are contributing to improved outcomes for patients: Example of Myeloma

RELATIVE SURVIVAL RATE FOR MULTIPLE MYELOMA PATIENTS SOARS BETWEEN 2001 AND 2014



Between 2001-2014, survival rates in multiple myeloma more than doubled. During this period there were FDA approvals of 4 new innovative drugs

CHANGE IN 5-YEAR SURVIVAL RATES FROM 1990-2013



5 year survival rates for multiple myeloma (from 1990-2013) have increased more than four times faster than for other cancers

Source: Celgene, "Value and Innovation, 2018" report. Top chart: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014; Bergsagel P. Where We Were, Where We Are, Where We Are Going: Progress in Multiple Myeloma. ASCO 2014 Educational Book; National Cancer Institute. Drugs Approved for Multiple Myeloma and Other Plasma Cell Neoplasms; Bottom chart: National Cancer Institute, SEER Cancer Statistics Review 1975-2014.

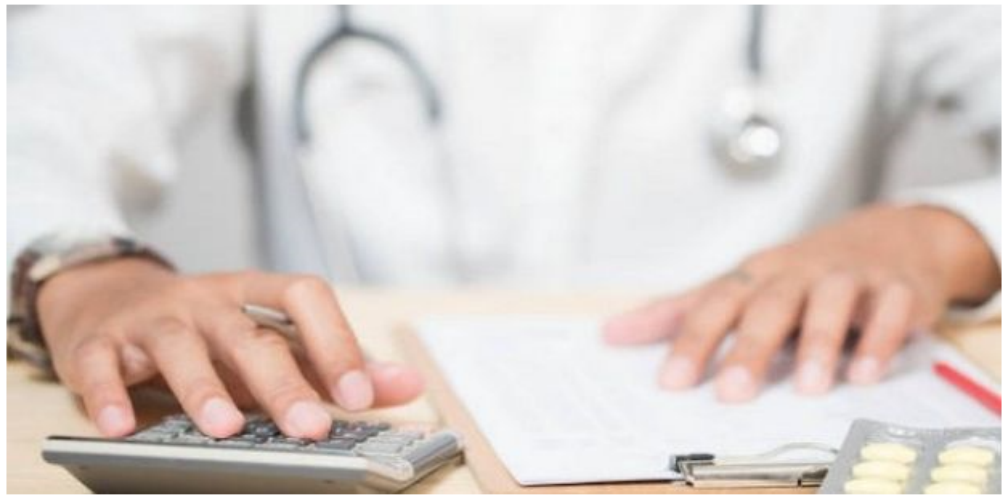
Innovation is delivering benefits, but concerns remain around affordability of modern medicines

Health: Warning over sustainability of healthcare spending

Review says health service consistently fails to manage within its budget allocation

Tue, Oct 9, 2018, 16:30 | Updated: Tue, Oct 9, 2018, 19:50

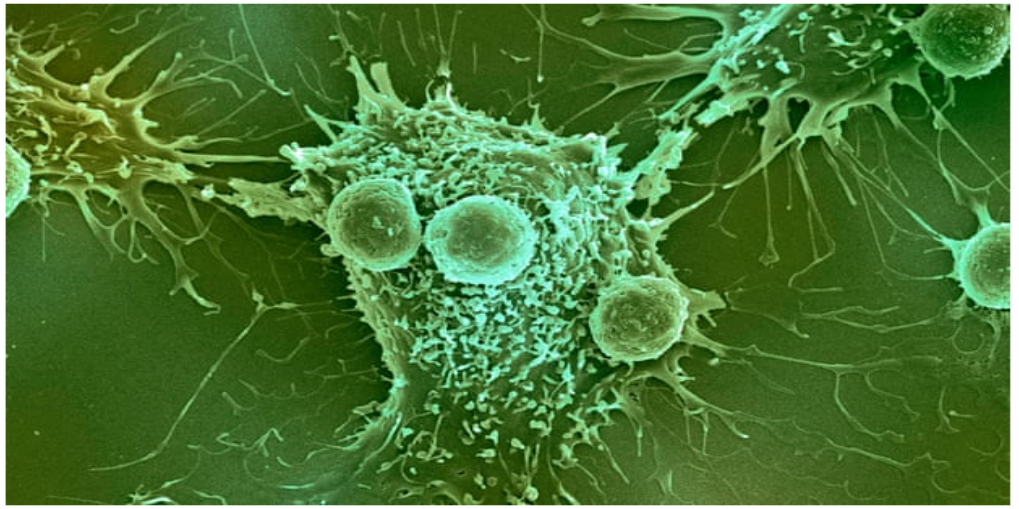
Martin Wall, Paul Cullen



A spending review has criticised the HSE for 'a re-occurring trend' of significantly increasing recruitment toward the end of the year. Photograph: Getty

NHS preparing to offer 'game-changing' cancer treatment

Health service chief calls for affordable access to CAR-T, which modifies immune system to destroy cancer cells



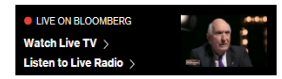
Economics

A Breakthrough Cancer Drug Has Been Approved. Now Comes the Battle Over the Price

By James Paton

September 12, 2018 6:00 AM CEST

- Drugmaker introduces Kymriah at 320,000 euros in Germany
- CAR-T cancer treatment was approved in Europe last month



OUR APPROACH TO NAVIGATING ACCESS CHALLENGES IN EUROPE

We Aim to Demonstrate Value to Patients & Society by…

Engaging with ALL stakeholders to understand their needs

Generating patient-relevant clinical and real-world evidence supported by a robust economic rationale

Securing reimbursement from HTA Agencies & Payers

Janssen Approach to Pricing

Three guiding principles drive our pricing decisions.

We deliver **local value** by collaborating with payers and governments to offer **accessible and affordable** medicines, fueling **sustainable innovation.**

Local Value

The value our medicines bring is in **improving the lives of patients and transforming their health for the future.**

We strive to deliver transformational medicines that improve the lives and health of patients. Our medicines help people live longer and improve their quality of life. We work closely with payers such as governments, insurers and other local stakeholders to negotiate the price of our medicines based on their local value — prioritizing health outcomes and the impact our medicines have on a specific society and economy.

https://www.janssen.com/about/access-pricing-principles

Janssen Approach to Pricing

Accessible and Affordable

Through active collaboration, we make our medicines readily **accessible and affordable** for patients and health systems in accordance with specific reimbursement systems and legal guidelines of local communities.

Countries and health systems around the world differ in how they pay for medicines and make them accessible to their citizens. Knowing this, we work with governments and payers, as early as the law allows, to discuss coverage, accelerate availability and provide the best possible access to our medicines. After all, medicines only deliver value when they reach patients who need them.

https://www.janssen.com/about/access-pricing-principles

Janssen Approach to Pricing

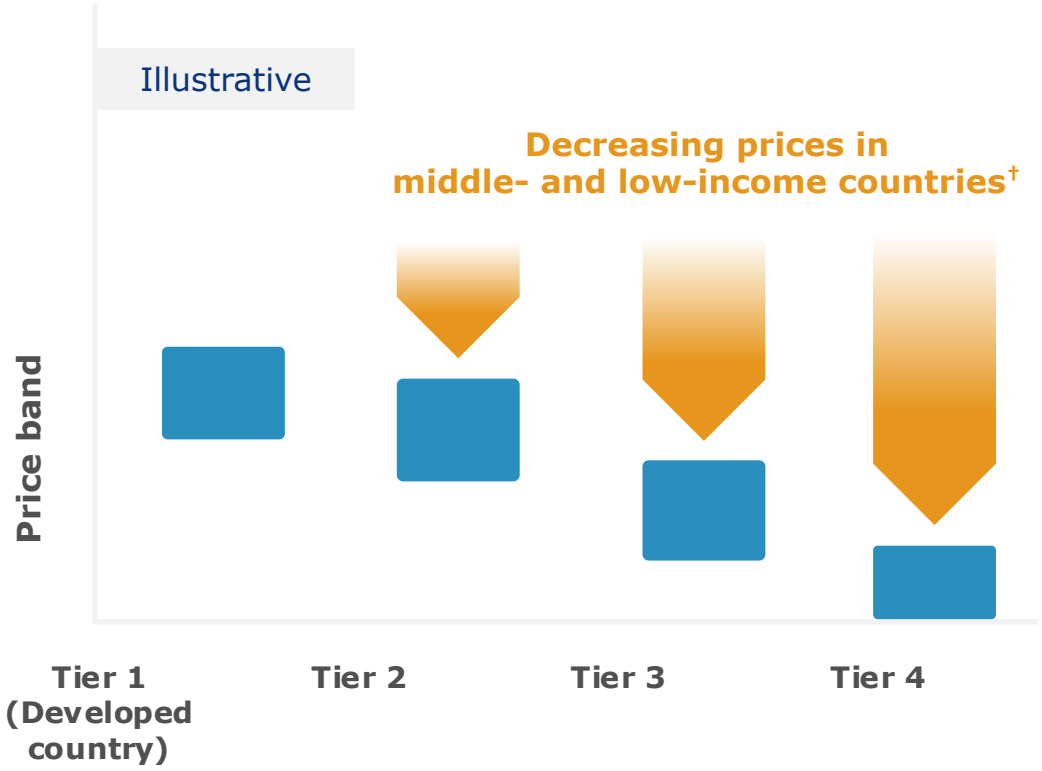
Sustainable Innovation

Sustaining the discovery, development and delivery of **transformational medicines is necessary to improve lives for current and future patients in need.**

When we succeed at delivering valuable medicines with transformational outcomes, individual patients thrive, and families, countries, communities and societies flourish. Enabling investment in innovation to discover, develop and deliver medicines provides significant benefit to patients today and tomorrow. Fair pricing for valuable therapies fuels the next breakthroughs and cures.

https://www.janssen.com/about/access-pricing-principles

Equity-based tiered pricing approaches are used to support access in lower and middle income countries



† Equity-based tiered pricing facilitates price variability across the world, with higher prices in countries with higher income and lower prices in lower income countries and where the burden of disease is greatest

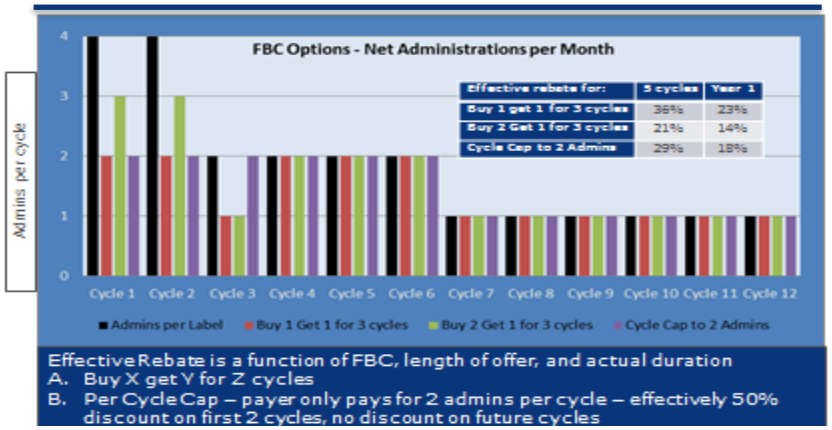
IRP = external reference pricing

Janssen strives to ensure local affordability

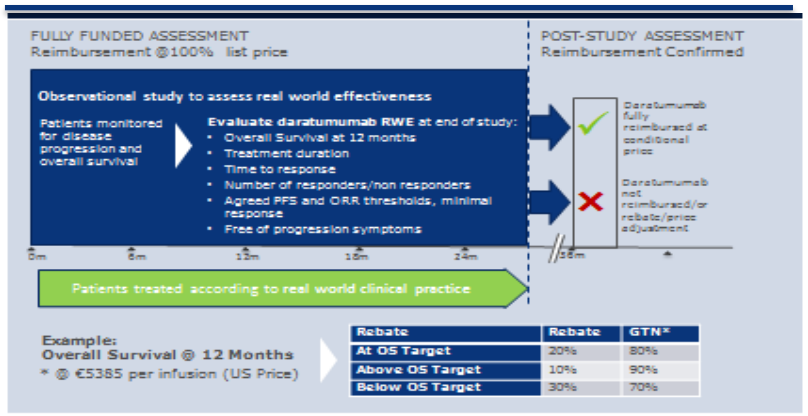
- Janssen uses tools such as equity-based tiered pricing to help achieve access that is affordable locally
- However, extensive IRP use and parallel trade preclude explicit tiered pricing (based on list price) in Europe
  - So to ensure broader access to our medicines, we sometimes negotiate flexible pricing agreements
  - This means that we may agree to make our medicines available at different net prices across Europe, in line with local affordability
  - These agreements are confidential, although the process is transparent within each country

To secure access we create managed entry agreement archetypes that we discuss with payers: Example in multiple myeloma

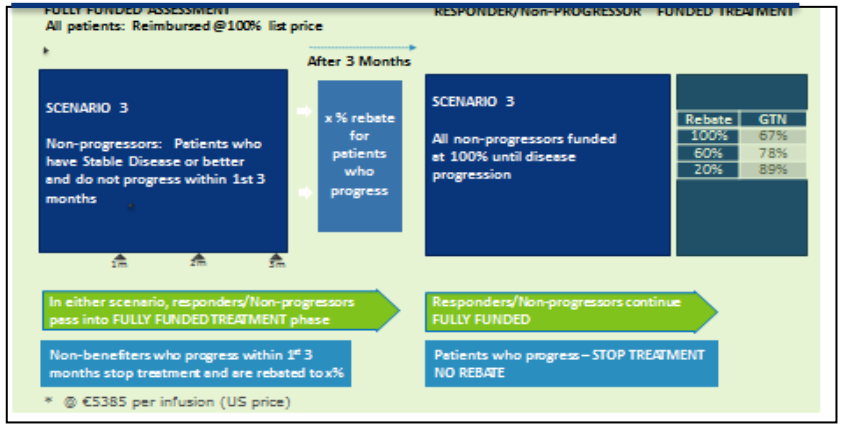
Agreements to manage initiation costs



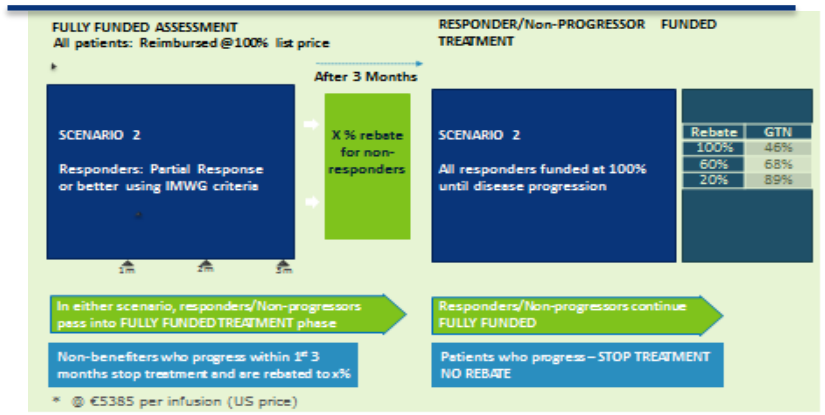
Coverage with Evidence Development



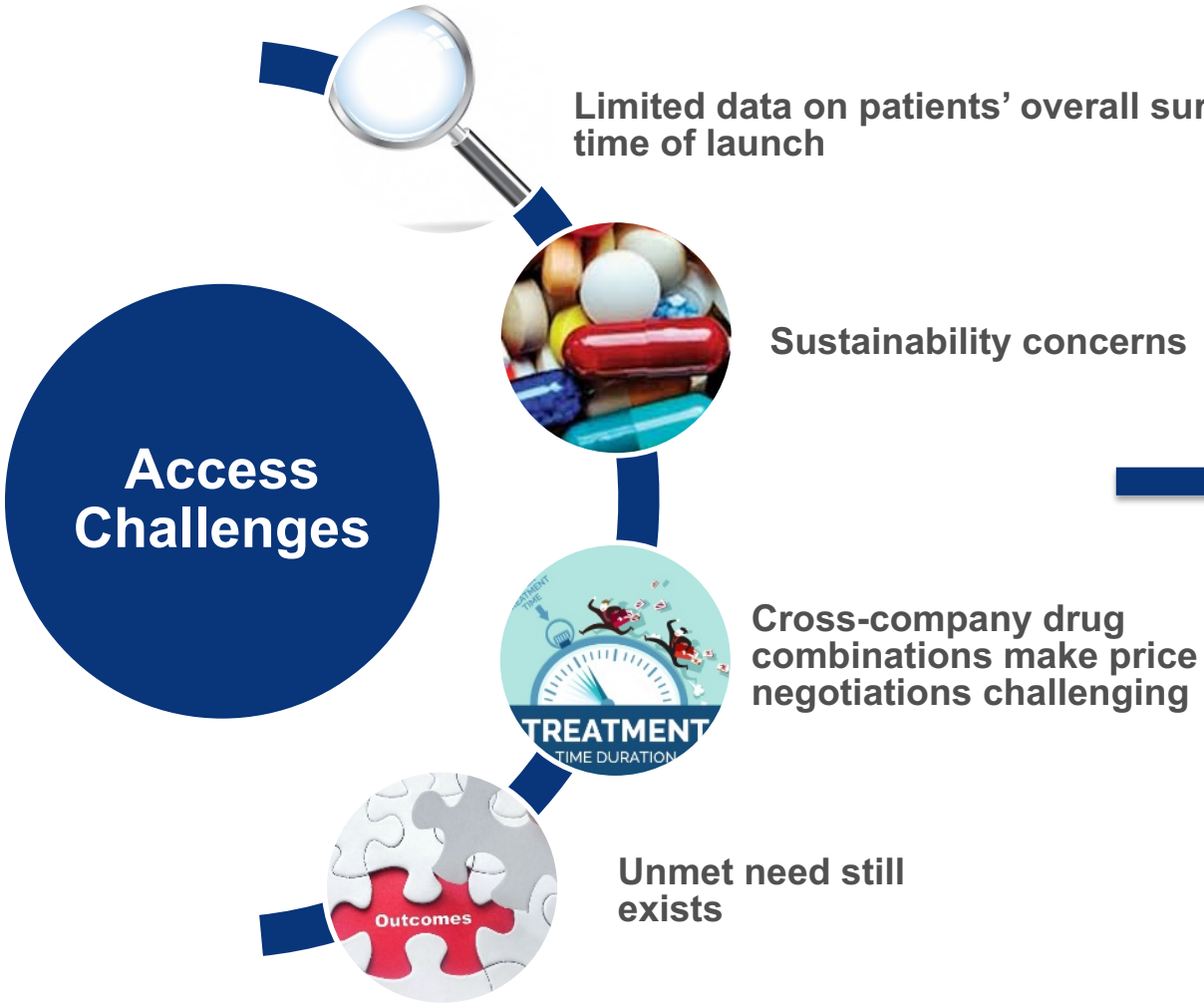
P4P: Rebate for Progressive Patients



P4P: Rebate for non-Responders



Emerging Access Challenges Require New Thinking where we need to involve Patients in the Debate



- Increased pricing flexibility e.g Drug Combinations, CAR-T
- HTA methodology reform
- A consistently strong patient voice in clinical research/HTA

HOW CAN WE WORK TOGETHER TO DO MORE FOR PATIENTS?

Janssen are constantly exploring ways to overcome reimbursement challenges in Europe in order to enable faster access for patients to cancer medicines

NEGATIVE IMPACT ON PATIENTS' LIVES

Delay submissions to HTA Agencies until OS data reaches statistical significance

Power all our clinical trials to demonstrate statistically significant OS

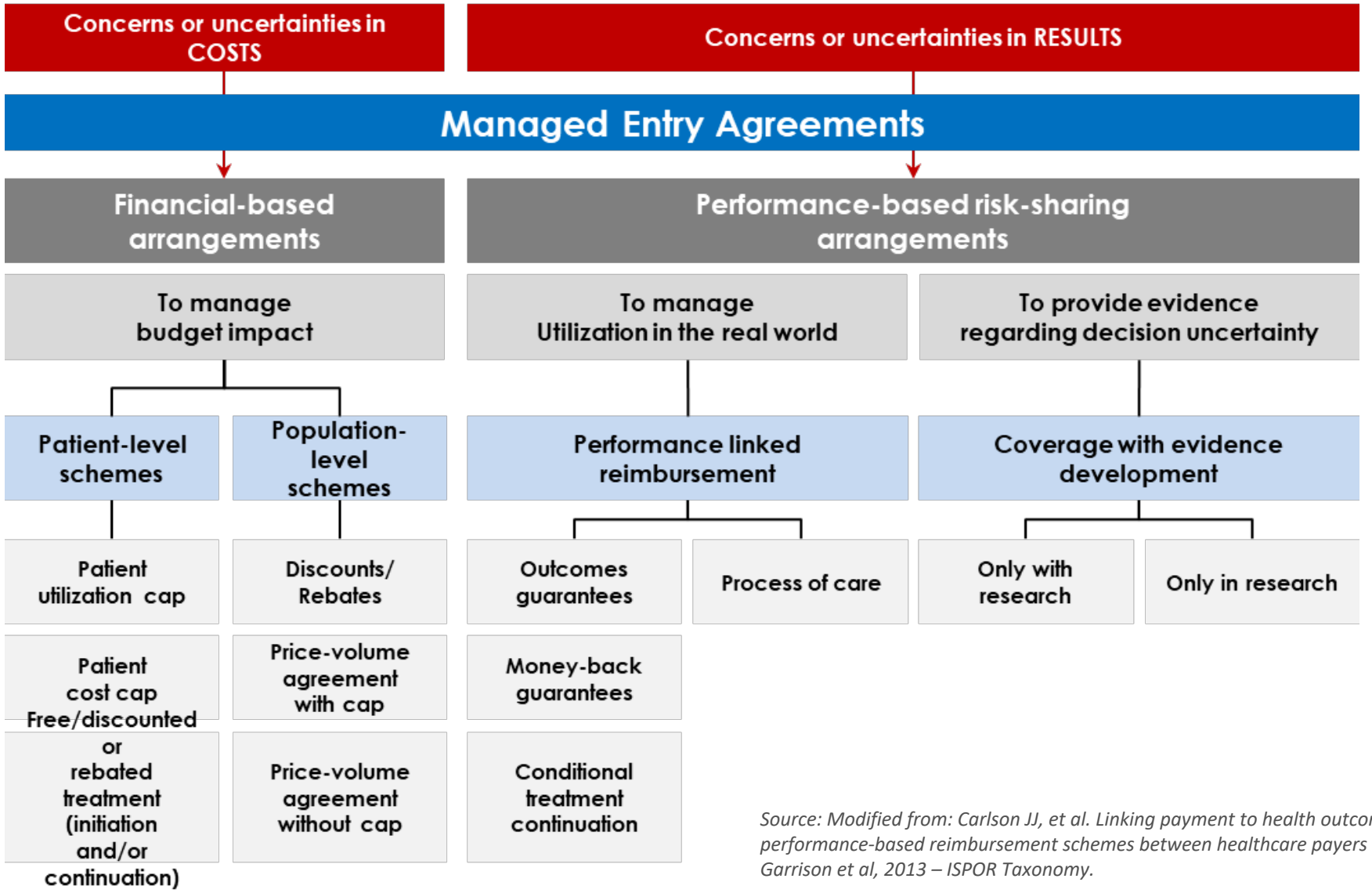
Identify valid alternatives or intermediate endpoints incl. PROs

Innovative managed entry agreements

Work with HTA Agencies and all stakeholders to modify current HTA methods & introduce key performance indicators at the country-level for patient access?

ACCESS & R&D COSTS

Greater Acceptance of Managed Entry Agreements Across Europe Is Needed to Manage Affordability Concerns



Source: Modified from: Carlson JJ, et al. Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. Health Policy (2010); Garrison et al, 2013 – ISPOR Taxonomy.

Engagement & Collaboration with all Stakeholders is Critical for Access

Patients and Caregivers

Nurses and Doctors

European Medicines Agency

HTA

HTA Agencies

Health Care Payers

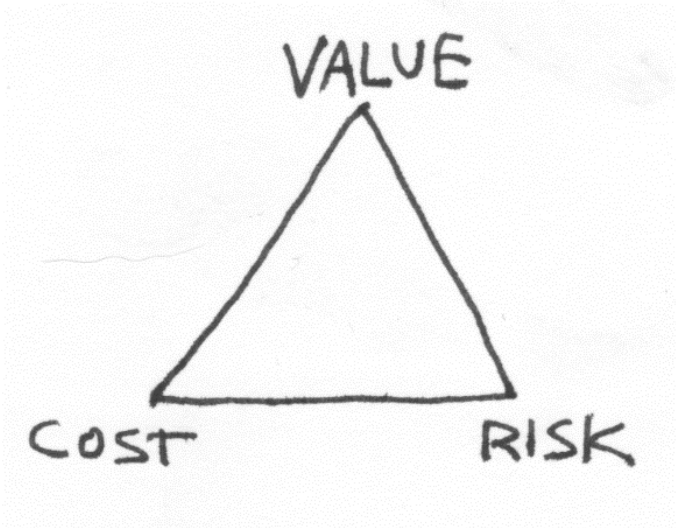
DESIGN FOR ACCESS

To ensure we represent the patient voice, we need to do more:

Patient Insights

Gather continuous insights from patients on their disease and treatment

Work together on Patient Evidence Strategies



Trusted Partnerships

Forge a closer collaboration with patients and patient advocacy organizations to co-develop solutions for access and the patient experience

How Patient Engagement Can Foster Access Through Improved Affordability?

Access & Affordability Considerations in the Pharmaceutical Industry

Dearmaid McDonald, Lead Organiser, Just Treatment

Thursday 24 September 2020 from 15.00 to 17.00 CET

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PATIENT FOCUSED
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Patients need to engage where it matters - where the rules of the game get written.

DIARMAID MCDONALD
LEAD ORGANISER

just TREATMENT

just TREATMENT

"THE HIGH COST OF THE MEDICINES MEANT I HAD TO WAIT THREE YEARS TO BE TREATED"

CLARE
in London

just TREATMENT

"IF COMPANIES CANNOT OFFER FAIR PRICES WE SHOULD TAKE ACTION TO COMPEL THEM TO DO SO"

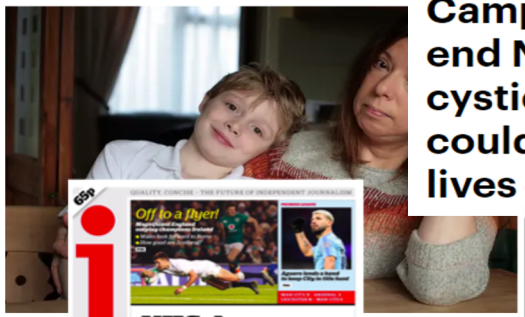
SIMON
in London

just TREATMENT

Calls for action on patients d
£100,000 cystic fibrosis drug

Exclusive: Ministers urged to consider revoking patent extending Orkambi, which NHS cannot afford

The patients facing shorter lives due to drug's expen



▲ Luis Walke
Orkambi. Pho

has been campaigning to get h

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Campaigners urge the Government to end NHS price war over £105,000 cystic fibrosis 'wonder drug' which could extend thousands of children's lives

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ust end' over cystic fibrosis
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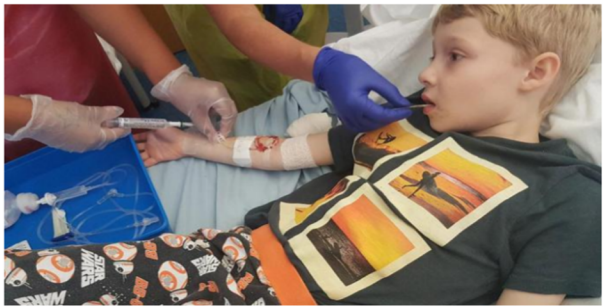
THE TIMES

‘Hundreds die’ after being denied cystic fibrosis drug

Chris Smyth, Health Editor

February 5 2019, 12:01am,
The Times

Health | NHS | Politics | UK politics | Conservative Party



The declining health of Luis Walker, eight, has been heartbreaking to watch, his mother, Christina, said
CHRISTINA WALKER/PA

Ministers urged to intervene over ‘unaffordable’ cystic fibrosis drug

Exclusive
Orkambi can extend lives but firm refuses to cut £105,000 price tag

Sarah Boseley
Health editor

Campaigners are calling on the government to intervene urgently over the cost of a cystic fibrosis drug that could extend the lives of thousands of people but has been priced by manufacturers at more than £100,000 per patient per year, which the NHS cannot afford.

For more than two years an emotive battle has been raging over Orkambi, which tackles the cause of cystic fibrosis. Previously, those with the genetic disorder did not often survive to adulthood and even today, half will die before the age of 32. Orkambi could extend lives, but negotiations over the price the NHS should pay have been in stalemate since July, with the pharmaceutical company that makes it refusing to reduce the price.

Health providers all over the world are struggling with the high price of new medicines, which has led to anger and campaigning, particularly over cancer drugs. The NHS has forced down prices by requiring drugs to go through a cost-effectiveness appraisal and capping the price it will pay.

Vertex, based in Boston in the US, has priced the drug at £105,000 per patient per year, which NHS England says is unaffordable. Vertex turned down its offer in July of £5000 over

‘My little boy has got an infection he may never eradicate’
Christina Walker
Mother of Luis

five years for access to Orkambi and its other cystic fibrosis drugs, saying it was inadequate and unfair. It has also withdrawn a newer and more effective drug, Symkevi, from the approval process, which means it will not be available to any NHS patients.

Campaigners have said the company earned $2.5bn (£1.9bn) in 2018 from sales of Orkambi, which was discovered with the help of funding from a charity, as well as taxpayers’ money from the US National Institutes of Health. Vertex paid $3.3bn for the patents. Its chief executive, Jeffrey Leiden, was paid at least $17.2m in 2017, according to filings reported by Reuters last year.

Christina Walker, whose eight-year-old son, Luis, has

Sunderland: Nissan cites

What is the purpose of patient engagement?

What influences access?

Cost
Price
Value

just TREATMENT

What is the purpose of patient engagement?

What influences access?

Cost
Price
~~Value~~

just TREATMENT

What influences high prices and undermines affordability?

Companies will seek to maximise profits for their shareholders. This is not a controversial statement.

- IP and other monopoly protections
- R&D incentive system
- Tax rebates and credits
- Price control mechanisms
- Financialisation of the pharmaceutical industry
- International trade agreements
- Public perception and reputation risk management

just TREATMENT

THE SUNDAY TIMES

Date: 20 September 202
Page: 14

NLA eClips

Patient charities discreetly take Big Pharma cash

Campaigns are failing to report millions they receive from drug firms

Andrew Gregory Health Editor

Hundreds of health charities are failing to declare millions of pounds received from the world's largest drugs companies. Pharmaceutical companies are pouring cash into patient groups that lobby for new treatments – in many cases the medicines marketed by the same donors.

The Charity Commission, the official regulator, urged charities last year to be "transparent" about cash received from drug companies to "protect their integrity". The warning came after it was revealed that Pain UK, a charity lobbying

National Institute for Health and Care Excellence, which decides on treatr the NHS will offer. The new study gests many have opaque financial tionships with the companies prov the treatments.

Previous research found that patient groups involved in the app of drugs or devices for use in the N England had received money from r facturers that they had not declarec

Decision-makers are some unaware of these conflicts of int The charities risk being used as vel to promote useless, expensive or

NHS

Patient groups assessing NHS drugs receive undeclared industry funds

Study calls for rules to be tightened over disclosure of money received from drug makers

How do we engage in a way that makes chances of access and affordability better for all patients?

just TREATMENT

Interventions that make monopolies, not patients the things put at risk

just TREATMENT

Demonstrate that the rules of the game are what is preventing access

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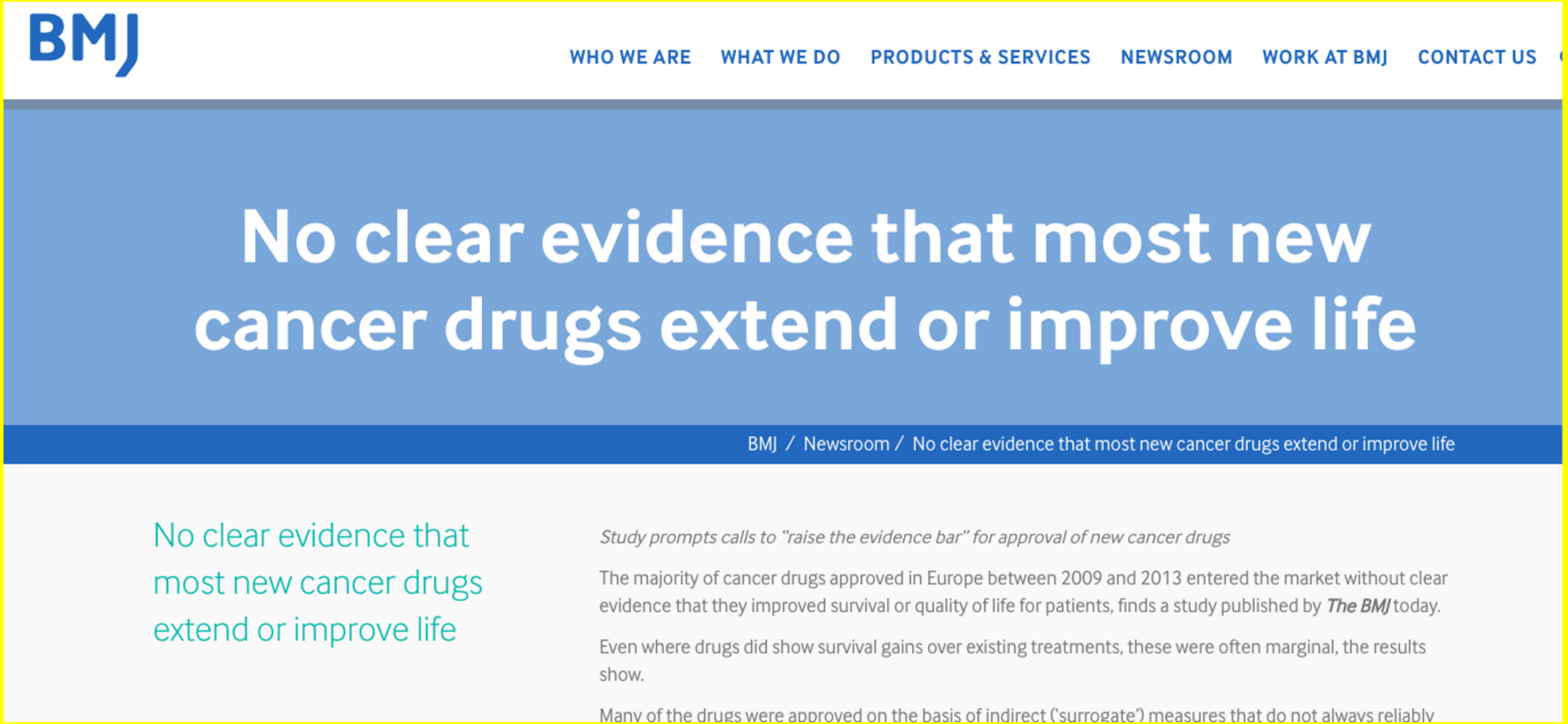

Demand a better innovation model

just TREATMENT

diarmaid@justtreatment.org
justtreatment.org
@JustTreatment

just TREATMENT

How Patient Engagement Can Foster Access Through Improved Affordability?

Access & Affordability Considerations in the Pharmaceutical Industry

Prof. Zoltán Kaló, Center for Health Technology Assessment, Semmelweis University

Thursday 24 September 2020 from 15.00 to 17.00 CET

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European Patients' Academy on Therapeutic Innovation

PATIENT FOCUSED
MEDICINES DEVELOPMENT

What science knows about access and affordability

Zoltán Kaló

Professor of Health Economics

1) Center for Health Technology Assessment, Semmelweis University
2) Syreon Research Institute

Evidence gaps about access and affordability

- **Indicators of limited patient access**
  1. **Time to reimbursement – well documented, but less important**
  2. **Access barriers after reimbursement – more important, bur poorly documented**
- **Impact of implementing value judgement in policy decisions**
  1. **Increased transparency and consistency of policy decisions**
  2. **Potential access barrier for high cost therapies in higher income countries**
  3. **Potential access barrier for all technologies in lower income countries**
- **Impact of extending value frameworks in the value judgement of health technologies**
  1. **Patient access**
  2. **Affordability → Opportunity cost**

Evidence gaps about access and affordability

- **Impact of price transparency**
  1. **Patient access in lower vs. higher income countries**
  2. **Free-ridership and race to the bottom**
- **Impact of transparency on development costs**
  1. **How to translate global data to local value judgement and price**
- **Impact of policy solution to facilitate affordability and sustainability of health care financing → access barriers**
  1. **Real world health gain**
  2. **Equity in access**
  3. **Perverse incentives (e.g. informal payments)**

Patient centricity of value judgement to health care decisions

Component 1: Patient engagement at different levels

- Macro level: health policy regulations, reimbursement decisions, HTA, clinical guideline development
- Meso level: hospital decisions
- Micro level: shared decision-making

Component 2: Patient centric value judgement

- Patient experience
- Burden on households

Differences in health care systems

Solutions to facilitate to patient access in high income countries may not be transferable to lower income countries due to

- more limited resources
- inefficiency of health care systems
- limited compliance of physicians with clinical guidelines as a consequence to perverse incentives
- inappropriate sales, marketing and market access practices of pharmaceutical companies
- less tradition and willingness for transparent and evidence informed decisions by payers and policy-makers
- brain drain of educated professionals and patient experts
- inefficiency of patient representations in health policy decisions (partly related to inappropriate funding models or patient organisations)
- international policy research projects have limited coverage to lower income countries

Takeaway messages
What's next?

Tamás Bereczky, Training Coordinator, EUPATI

Thank you!

Do you want to get in touch with us?

- info@eupati.eu
- www.eupati.eu
- @eupatients

Patient Engagement open forum Into Reality

Let's work together to spread the word!

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