How Can Patient Engagement Foster Access Through Improved Affordability?

September 24th from 15.00 to 17.00 CET
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!
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
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
   • Diarmuid 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.
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)

**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 (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 EFSCP)

**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
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.\(^2\)

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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

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3. [https://www.accessdata.fda.gov/drugsatfda_docs/label/2015/204671s002lbl.pdf](https://www.accessdata.fda.gov/drugsatfda_docs/label/2015/204671s002lbl.pdf)
What does it cost to profitably mass-produce SOF?

**Sofosbuvir**

- **Cost of API = $700/kg**
- API needed per person = 33.6g (400mg x 84 days)
- API per 12 weeks = $23.52
- Formulation = $0.01/tablet, excipient cost per tablet (API x 2 x $2.63)
- Formulated drug = $24.54
- Profit margin (10%), tax on profit (27%)
- **Final generic price = $27.65**

Slide courtesy of Dr Andrew Hill
What does it cost to profitably mass-produce DCV?

C. Daclatasvir

Cost of API = $600/kg

API needed per person = 5.04g (60mg x 84 days)

API per 12 weeks = $3.02

Formulation = $0.01/tablet, excipient cost per tablet (API x 2 x $2.63)

Formulated drug = $3.89

Profit margin (10%), tax on profit (27%)

Final generic price = $4.38

Slide courtesy of Dr Andrew Hill
Prices for 12 weeks of SOF/DCV by Country

- USA (NADAC)
- Denmark
- Norway
- Germany
- United Kingdom (Quebec)
- Sweden
- France
- Argentina
- Saudi Arabia
- Spain
- Brazil
- Australia
- Thailand
- Egypt
- India
- Estimated

Slide courtesy of Dr Andrew Hill
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
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, 16:20  Updated: Tue, Oct 9, 2018, 19:59

Martin Wall, Paul Callon

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

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

Tier 1 (Developed country) Tier 2 Tier 3 Tier 4

Illustrative

Decreasing prices in middle- and low-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

- Limited data on patients’ overall survival at time of launch
- Sustainability concerns
- Cross-company drug combinations make price negotiations challenging
- Unmet need still exists
- 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

Engagement & Collaboration with all Stakeholders is Critical 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
Patients need to engage where it matters - where the rules of the game get written.

DIARMAID MCDONALD
LEAD ORGANISER

just TREATMENT
"The high cost of the medicines meant I had to wait three years to be treated."

CLARE
In London

"If companies cannot offer fair prices we should take action to compel them to do so."

SIMON
In London
Campaigners urge the Government to end NHS price war over £105,000 cystic fibrosis 'wonder drug' which could extend thousands of children's lives.
What is the purpose of patient engagement?

What influences access?

Cost  
Price  
Value
What is the purpose of patient engagement?

What influences access?

Cost
Price
Value
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
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 treatments the NHS will offer. The new study suggests many have opaque financial relationships with the companies providing the treatments.

Previous research found that patient groups involved in the approval of drugs or devices for use in the NHS had received money from manufacturers that they had not declared.

Decision-makers are sometimes unaware of these conflicts of interest. The charities risk being used as vehicles to promote useless, expensive or ineffective treatments.

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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?
Interventions that make monopolies, not patients the things put at risk.
Demonstrate that the rules of the game are what is preventing access
No clear evidence that most new cancer drugs extend or improve life

Study prompts calls to "raise the evidence bar" for approval of new cancer drugs

The majority of cancer drugs approved in Europe between 2009 and 2013 entered the market without clear evidence that they improved survival or quality of life for patients, finds a study published by The BMJ today.

Even where drugs did show survival gains over existing treatments, these were often marginal, the results show.

Many of the drugs were approved on the basis of indirect ('surrogate') measures that do not always reliably
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
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, but 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
Let's work together to spread the word!

#PEOF2020

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@eupatients
@PFMDwithPatient