Access to Innovative Drugs
Nathalie Moll, Director General

13 October 2022

What is driving the debate
Availability of medicinal products in Europe

The time to availability is the days between marketing authorisation and the date of availability to patients in European countries

European Union average: 76 products available (45%), Limited availability (14% of all products). Not submitted did not submit complete information on restrictions to available medicines meaning LA* is not captured in these countries. In most countries availability requires a listing of access to the reimbursement scheme. In the EU, Belgium, Denmark, France, Germany, Italy, Spain, Sweden, and the UK, where some hospital products are not covered by the general reimbursement scheme. *Countries with asterisks did not complete a full dataset and therefore availability may be unrepresentative. **In Spain, the WAIT analysis does not identify those medicinal products being accessible earlier in conformity with Spain's Royal Decree 17/2008 regulating medicines in Special Situations.
Delays and time to availability
All medicinal products

The time to availability is the days between marketing authorisation and the date of availability to patients in European countries

Delays and unavailability

We share the goal of fast, equitable and sustainable access to treatments and recognise the disparities and delays in access for patients across Europe. Millions of people across Europe are not always able to access the scientific breakthroughs when they need them. Data from EFPIA’s Patients W.A.I.T Indicator show that market authorisation and patient access can vary from three months to 2.5 years, depending on the country and region. Addressing these issues requires a shared, evidence-based understanding of the root causes of barriers and delays in access to treatments.
What is the evidence?

- **Wide variations** in availability and delays across Europe
- Although access to **oncology medicines** appears to be improving, access to **orphan medicines** continues to vary considerably across EU Member States
- Even **within one country**, patients can get access to some medicines almost immediately, and wait years for others
- There is little evidence that **delays** are reducing – rather the contrary
10 key factors causing delays in patient access to new cancer medicines in Europe.

10 interrelated factors that cause access to medicines to be delayed:

- Time prior to marketing authorisation
  1. The speed of the regulatory process
  2. Accessibility of medicines prior to marketing authorisation
- The price and reimbursement process
  3. Initiation of the process
  4. The speed of the national timelines and adherence
- The value assessment process
  5. Misalignment on evidence requirement
  6. Misalignment on value and price
- Health system readiness
  7. The value assigned to product differentiation and choice
  8. Insufficient budget to implement decisions
  9. Diagnosis supporting infrastructure and relevance to patients
- Delays from national to regional approval
  10. Multiple layers of decision making processes
Strong commitment from industry to improve patient access to treatment

**Equity Based Tiered Pricing**

**Tiering framework**
- **Upper tier**
  - Companies comply with EBTP and set list prices for markets based on their own value-based pricing principles
  - Price negotiations with Member States under application of non-extraterritoriality
- **Lower tier**
  - Companies comply with EBTP and set list prices for markets based on their own value-based pricing principles
  - Price negotiations with Member States without factoring ERP

**EBTP implications**
- The final net prices and value-based agreements are determined at the MS level
- The “best price” from the upper tier stipulates the maximum price level for the lower tier

**EFPIA’s Access Portal**
- The Portal tracks patient access to newly launched medicines across European markets and highlights root causes behind access delays

**Commitment to File**
- Industry commits to filing of P&R applications post EU authorisation in all EU-27 Member States as soon as possible and no later than two years, provided that local systems allow it

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**A shared Equity Based Tiered Pricing**

Pricing of medicines based on countries’ ability to pay (using gross national income in purchasing power parity) to improve patient access (speed and availability) across Europe

**Key principles – to be co-created with relevant stakeholders**

- The concept of solidarity is fundamental: wealthier Member States should not benefit from lower prices ought to be available, in the interests of patient access, to less wealthy countries
- Anchored in value-based pricing: pricing of medicines based on value they deliver to patients, healthcare systems and society
- Part of a broader response to improve access and affordability
- Application to a product needs flexibility
- Role may evolve over time, as the differences between countries change

Win-win for patients (reduced delays, improved availability), Member States (price in line with value and ability to pay), EU institutions (better access to medicines) and industry (products in more markets)
The Joint EU HTA has the potential to accelerate patient access

**Opportunity**
- Common scientific foundation for P&R decisions for all Member States
- Reduction of duplication of evidence submissions
- Reduction in resource requirements for smaller countries
- Potential to accelerate patient access to new medicines across European markets

**Challenge**
- Requires creation of a genuine new system that is applicable and supported by all national HTA bodies
- Needs to be sufficiently resourced
- Needs to include adequate level of manufacturer involvement
- Needs to ensure adequate level of data confidentiality

Challenges remain - variation in evidence requirements is a barrier to patient access

**Level of acceptance of evidence characteristics**

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Inconsistency of evidence requirements between EMA and HTA and across national HTA bodies creates duplication and patient access delays.

Meeting a wide range of evidence requirements across WHO European Region’s 53 countries is a burden for MAH especially in rare disease areas.
Industry and stakeholder initiatives are leading to progress in patient access

We have successfully worked with healthcare systems across the EU to enable patient access to our CAR-T treatments, using managed access agreements, including:

- **Outcome-based agreements**: Payment for treatment is agreed subject to meeting individual patient level outcomes.
- **Value-based agreements**: Price for treatment is agreed subject to providing additional longer-term clinical evidence for cohort patient population.
- **Flexible payment**: Payment in instalments to support cashflow and affordability for the healthcare system.
- **Price volume agreements**: Price for treatment is agreed subject to a quantity of product ordered.
- **Price by indication**: Price for treatment is agreed across various indications taking into account value of indications.

Novel payment models can improve access to innovation

- **Estonia**: Indication-based pricing: using data infrastructure for disease areas with small patient populations.
- **United Kingdom**: Outcomes-based pricing: NHS paid for patients with a complete or partial response after four cycles of treatment.
- **Netherlands**: Indication-based and outcomes based pricing: implemented with sick funds and individual hospitals.
- **Belgium**: Indication-based and outcomes based pricing: physicians required to meet criteria to initiate, prescribe or discontinue in to a sick fund database.
- **Germany**: Outcomes-based pricing: rebates given from company if patients die from disease in given period.
- **Switzerland**: Combination pricing: payers provide a refund to companies based on the difference in price of the sum of the individual medicines and that of the combination. Breast cancer and myeloma.
- **Spain**: Outcomes-based model: companies rebates. payers up to 100% for suboptimal or non responder patients with CML.
- **Denmark**: Subscription payment model: payers pay a subscription for an unlimited number of patients to be treated with current or pipeline CF treatment over defined period.
- **Italy**: Over-time model: for CAR-Ts instalments at administration, six months and a year and only if shown to be effective. National registries allow for outcomes-based reimbursement, indication and combination pricing.
Varying health priorities across Eastern European Countries

Highest disease burden and associated health priorities vary across Eastern European countries:

- **Ukraine** – leading cause of death 2022: CVD, followed by COVID-19
- **Slovakia** – leading cause of death 2022: Coronary Heart Disease followed by Dementia
- **Romania** – leading cause of death 2022: Cerebrovascular disease & high blood pressure
- **Serbia** – leading cause of death 2022: CVD, followed by Cancer
- **Armenia** – leading cause of death 2022: CVD, followed by COVID-19
- **Hungary** – leading cause of death 2022: Cancer, followed by ischemic heart disease

We are on the right path, but further progress is needed – industry stands ready to partner with the stakeholder community

**Vision**
Every patient in need should have access to our medicines

**Journey**
We need to work together to address existing challenges – it’s a journey not a sprint

**Collaboration**
Industry is working with healthcare systems to find new flexible approaches to patient access

**Focus**
We need to find bespoke solutions tailored to countries’ need – top-down regional policy will not address individual challenges

**Framework**
Need to develop a framework of core principles that recognises the individual needs of countries and offers tailored solutions

**Solidarity**
Need for further solidarity and collaboration between countries to work together in RWE collection to avoid duplication and enable joined learning
## Lessons learnt from Joint Procurement Initiatives

### Challenges posed by Joint Procurement Initiatives

1. **Gilead provided the only available treatment for COVID through the JPA across the EU during the pandemic.**
2. **Despite the urgency and lack of alternative treatment options, difficulty by MS to find agreement on need for collaboration, supply and allocations.**
3. **Difficulty in reaching agreement will be even greater in non-crisis times for treatments for routine use.**
4. **Similarly, Beneluxa initiative has not resulted in many agreements yet even given that it involves a small number of similar countries.**
5. **It will be even more difficult to reach agreement if 27 MS are involved of varying sizes and eco strength.**

### Suggested principles for Joint Procurement Initiatives

1. **Overall objective should be enhancing and accelerating patient access.**
2. **Should not lead to additional market access barriers nor duplicate national negotiation processes.**
3. **Should be confined to countries of similar economic and health-related needs.**
4. **Industry participation in the initiative should be voluntary.**
5. **Confidentiality of pricing and reimbursement agreements should be guaranteed.**

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### Aligning price, value and budget

[Images of laboratory equipment and scientific research]
Value-based approach to pricing

The goal of pricing of pharmaceutical innovations is to ensure that patients can access medicines in a way that is sustainable for healthcare systems, whilst also supporting a sustainable stream of innovation that delivers continuous improvements in the treatment options available for patients. Prices send signals to innovators about where to focus their R&D efforts, as well as determine the overall level of investment in health and expected value of innovation in the pipeline. A value-based approach to pricing is based on the principle that prices should reflect the value of a new medicine to 1) patients, 2) health systems and 3) society versus the current standard of care.

A value-based approach to pricing can deliver the triple win of:

- Innovation
- Patient access
- Health system sustainability

**Why:** for innovative medicines, prices are set in negotiation between a monopoly seller and a monopsony buyer – these negotiations need to be guided by some set of principles on how prices should be set

**Principle:** Prices should reflect the value of a new medicine to patients, healthcare systems & society, versus the current standard of care.

Many countries in Europe have introduced elements of value-based pricing, but there are still significant gaps and barriers which disrupt the alignment between value and price.
What is value?

Value to patients:
- Improvements in patients’ health, e.g. increased survival, quality of life, functional status and ability to take part in work and daily/social life
- Improvements in process of care, e.g. a pill compared to a transfusion, for example, may save them discomfort, as would decreased travel time to a healthcare provider.

Value to healthcare systems:
- Replacement of a more expensive (or equally expensive but less effective) alternative
- Prevention of complications that would lead to hospitalisation/emergency care
- More efficient patient pathway
- Slower disease progression which means less resources needed for intensive care or social care
- New treatment modalities that allow for home care instead of hospital care

Value to society:
- Patients returning to work
- Lower costs for sick leave or other social benefits
- Reduced burden on informal carers
- Heard immunity from vaccination

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EU member states’ consideration of value elements in health technology assessment processes

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<tr>
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<td>Indirect non-medical costs</td>
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<td>Reducing unmet need</td>
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<td>Treating rare diseases</td>
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Pricing approaches & price control measures used by EU member states

<table>
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<td>Therapeutic referencing</td>
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All countries make:

• some provision to reflect the results of value assessment in their pricing and reimbursement decisions.
• use of other pricing approaches or price control measures that disrupt the alignment between value and price.

The most prevalent of these are external reference pricing & measures to control overall pharmaceutical expenditure, both of which are used in seven of nine countries.

What should healthcare system stakeholders do?

1. Ensure meaningful involvement of all stakeholders in value assessment
2. Enhance collaboration and share expertise across EU Member States
3. Develop a shared and holistic definition of value
4. Recognise qualitative evidence of value through deliberative processes

Enhance value assessment:
5. Fully embrace a value-based approach
6. Extend value-based pricing to the indication level

Improve the implementation of value-based pricing:
7. Use outcomes-based managed entry agreements to manage residual uncertainty
8. Enhance data collection infrastructure to allow for iterative assessments of value post-launch
9. Commit to ‘Equity Based Tiered Pricing’
10. Promote competition

Maximise the benefits of value-based pricing through complementary tools:
Broadening the perspective on affordability

Health care budgets are under pressure, resulting in reduced access to individually cost-effective therapies because the total cost of care outgrows the available budget. Taking a broader perspective across time and budgets can improve the affordability of pharmaceuticals and safeguard future patient access to valuable therapies.

The net pharmaceutical expenditure is an important, but smaller part of the total healthcare costs

*Net spending on pharmaceuticals represents between 8% and 24% of healthcare budget in 2018*

![Chart showing net pharmaceutical expenditure as a percentage of healthcare budget in 2018](chart.png)

Source: World Health Organization (WHO), 2018 (extracted on 14 November 2018); WHO data (extracted on 14 November 2018), OECD, 2019 (extracted on 14 November 2018). Notes: Drug spending includes medicines dispensed in both retail and non-retail sectors. Methods for estimating total drug spending on a net basis are detailed in the methodology appendix. Health spending from WHO database. Both the drug and health spend data were adjusted for population, Purchasing Power Parity (PPP), and GDP growth to represent in 2020 values. Czech Republic has low healthcare spend, contributing to the high % pharmaceutical spend.
Pharmaceutical expenditure is often small in comparison to societal cost of diseases

In Europe, medication accounts for only 7.6% of economic burden of CVD

Source: European Heart Network, 2017 report on European Cardiovascular Disease statistics: https://ehnheart.org/cvd-statistics.html#:~:text=Overall%20CVD%20is%20estimated%20to,care%20of%20people%20with%20CVD

Countries’ net pharmaceutical spending has remained ~15% of healthcare expenditure since 2000

Countries’ net pharmaceutical spending has been converging over the past 20 years

Source: World Health Organization (WHO), 2018 (extracted on 18 November 2021), WHO SHE 1.0 data (extracted on 18 November 2021); OECD, 2019 (extracted on 18 November 2021).

Notes: Drug spending includes medicines dispensed in both retail and non-retail sectors. Although drug spending data is available on a net basis, additional details with methodology and approaches to be included. Health spending from WHO database. Both the drug and health spending data are adjusted for population. Purchasing Power Parity (PPP) and GDP growth in prices of 2020 values.
Budget holders who view spend in silos will restrict access when savings are being made elsewhere

*Spending in one area can be directly counteracted by a decline in another*

Comparison of cardiovascular and oncologics real LC$ spend, 2000–2020

Perceived ‘budget busters’, such as Hep-C, had a short-lived impact which was outweighed by societal cost

*Even real spend on Hep-C treatments is offset by other healthcare and societal savings*
The pharma industry is launching more NASs than ever before, with the majority for smaller disease classes with high unmet need

*However, significant biosimilar and Gx approvals balance-out expenditure on innovative medicines*

**Profile of innovative medicines since 2000 by EMA approval year**

- 168 innovative approvals (2006-2010)
- 211 innovative approvals (2016-2020)


**Notes:** EMA EPAR list of authorised medicines includes all authorised medicines within historic data; human medicines approvals only, vaccines included. Innovative medicines is based on previous approval for the active substance, multiple indications only included upon initial submission, includes orphan medicinal products regardless of prior approval status. Key: * = Analysis accurate as of April 2021.

Cost-containment measures targeting innovative pharmaceuticals can exacerbate the affordability challenge

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<th>Health as investment</th>
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<td>Improvements in health outcomes</td>
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<td>Increase in productivity</td>
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<td>Increase in per-person income</td>
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<td>Increase in GDP per capita</td>
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Cost containment risks breaking positive feedback loop
To improve the collective affordability of therapies and pharmaceuticals, ability to pay needs to increase or costs need to decrease

**Affordability definition:**

\[
\text{Ability to pay} \geq \text{Costs}
\]

**Improve affordability:**

Increase ability to pay, however:
- Increasing health spending without reducing other government spending increases overall government spending.
- Increasing health spending by reducing other government spending creates difficult opportunity cost trade-offs.
- Shifting away from principle of universal healthcare is unlikely to be politically feasible or desirable.

Decrease costs, however:
- Pharmaceutical expenditures are primarily a necessary investment in better health outcomes.
- Sustainability of the economic model for developing innovative therapies is already under pressure.
- Share of pharmaceutical expenditure as a proportion of total healthcare expenditure has not increased.

**Taking a broader perspective across time and budgets helps to identify cost offsets – net savings for the system – that can contribute to solving the affordability challenge**

**COST OFFSETS**

*Savings or foregone expenditures created by the therapy*

**Time dimension**

- **Short term**
  - Pharmaceutical spending
- **Medium term**
  - Healthcare spending
- **Long term**
  - Beyond healthcare

**Budget dimension**

- Faster dismissals from care location
- Reduced need to revisit care location
- Increased ability to go back to work
Pharmaceutical spending can improve affordability by generating cost offsets over time

**Affordability across time**

1. Improving **affordability in the long-term** might require **investments in the short-term**.
2. Investments now pay off in **lower costs** in the future as **cost offsets** are realized.
3. Overall affordability improves if the upfront investment can be financed.

**Example: Haemophilia B gene therapy**

- Without gene therapy, patients with moderate to severe haemophilia B can cost health care systems more than $20 million over their lifetimes.
- A new gene therapy has been found with clinical effectiveness for up to 23 years, resulting in significant cost reductions over time—despite a multi-million price tag for the drug.

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Pharmaceutical spending can also improve affordability by generating cost offsets across budgets

**Affordability across budgets**

Example: Biologics for rheumatoid arthritis

- Disease-modifying antirheumatic drug biologics (bDMARDs) significantly reduce absenteeism from work and presenteeism at work.
- Society benefits from this through higher productivity and tax incomes, but these benefits are generally not included in cost-effectiveness assessments of new drugs.

**Example:** Biologics for rheumatoid arthritis

- Pharmaceutical spending in budget A can generate **cost offsets** for **another budget holder** in budget B, e.g., when a therapy:
  - Reduces the need for nursing care (at home).
  - Enables patients to return to the workforce sooner.
- When managed in **siloes** these cross-budget cost offsets are not valued by the budget holders resulting in **underinvestment** from a cross-budget, societal, point of view.
- If budgets are managed with a **cross-budget perspective**, cost offsets created are considered and **allocation is optimized**.
Five solutions can be used to take a broader perspective and realise long-term, cross-budget cost offsets that contribute to pharmaceutical affordability

- **Long-term horizon scanning & multi-year budgeting**
  - Increasing the time perspective for payors in planning and contracting helps to make more efficient assessments of new therapies that include cost offset considerations.

- **Societal value perspective in HTA**
  - Broadening the perspective of Health Technology Assessments to include the societal perspective helps to recognize impact of therapies beyond the care domain (e.g., productivity gains).

- **Innovative reimbursement agreements**
  - Innovative reimbursement agreements let payors manage risk and costs over a longer period of time and opens the door for health investments.

- **Integrated budgeting**
  - Merging siloed (pharmaceutical) budgets improves allocation decisions by payors as external effects and cost offsets in other budgets can be fully internalized in the decision making.

- **Social impact bonds for healthcare**
  - With impact bonds, effective therapies can be funded by third parties through performance-based contracts, creating a new funding source.

**Recommendations: Ensuring sustainable ATMP access for healthcare systems and patients**

- **Increase use of innovative payment models** that distribute costs over time
- **Maintain a collaborative environment** for developing innovative payment models through co-creation and shared learnings
- **Enhance horizon scanning**
- **Implement adaptive budget impact analyses**
- **Reconfigure budget silos**
Major revision of EU pharmaceutical laws upcoming and healthcare high up on the Brussels political agenda – how did we get here?

- **2016** Council conclusions on strengthening the balance in the pharmaceutical systems in the European Union and its Member States
- **2020-...** COVID-19, war, inflation and energy crisis
- **November 2020** Pharmaceutical Strategy for Europe
- **2023-...** Revision of the EU general pharmaceutical legislation and the orphan and paediatric regulations

Complementary EU and national responsibilities
Ensuring Europe's competitiveness & addressing the needs of patients

1. **General pharmaceutical legislation**: regulates authorisation, manufacturing, distribution and monitoring of medicines + provides regulatory protection to reward innovative medicines

2. **Orphan Medicinal Products + Paediatric Regulation**: complement the general pharmaceutical legislation – support the development of medicines in previously neglected areas)

1. **National reimbursement legislation**: regulates access and reimbursement of medicinal products

2. **Health care system readiness**: ensure future proof health systems
Simultaneously: Mind the gap
Europe’s research and development base is gradually eroding

Only through a future-proof regulatory framework, and a robust and predictable intellectual property and incentives ecosystem, can Europe become a true world-leader in medical innovation.

Access to Innovative Drugs
Nathalie Moll, Director General

Thank you!