efpia
European Federation of Pharmaceutical Industries and Associations

A shared approach to supporting Equity Based Tiered Pricing

Discussion document

New potentially curative treatments are being discovered with the potential to transform the lives of patients, the way we think, manage and resource healthcare. However, innovation only matters if it reaches patients when and where they need them. As illustrated by the most recent Patient W.A.I.T. Indicator Survey in 2021, the average time to reimbursement for innovative treatments across countries in the EU and the European Economic Area continues to be as long as 511 days, ranging from 133 days in Germany to over 899 days in Romania.[1]

Over the past two years, EFPIA has documented the drivers behind these delays and unavailability. EFPIA has published an assessment of the root causes of unavailability and delay (defined as length of time from European marketing authorisation to availability at Member State level) to innovative medicines, building on the long established WAIT analysis.[2] This recognises that there are patient access inequities within Europe, with significant differences across countries in the number of products that are available at a point in time and that the time taken prior to national reimbursement also varies significantly from one country to another. This analysis has gone further than in the past in setting out the multiple root causes for unavailability and delayed access, summarising five different categories and 10 root causes. The causes are rooted in the medicines access systems and processes in the Member States and the corresponding impact on commercial decision-making. These include a slow regulatory process, late initiation of market access assessment, duplicative evidence requirements, reimbursement delays, and local formulary decisions.[3] As the root causes are multifactorial, they can only be solved in partnership with the broader healthcare community including Member States.

The European Commission is currently preparing a revision of the EU Pharmaceutical Legislation and has put forward a range of proposals to address patient access inequalities across EU member states. This includes stepping up co-operation with and among Member States on the affordability of medicines. We understand that some of the proposals being discussed could introduce obligations for Marketing Authorisation Holders (MAHs) to market or supply all EU Member States. The industry has concerns regarding the use of regulatory tools designed for medicines authorisation being applied to address availability issues that are within the remit of Member States. Any requirement for MAHs to place a centrally authorised medicine on the market in the majority of Member States (including small markets) within a

1 https://efpia.eu/media/636821/efpia-patients-wait-indicator-final.pdf
2 https://efpia.eu/media/636822/root-cause-unavailability-delays-cra-report-april-2022-final.pdf
3 https://efpia.eu/media/636822/root-cause-unavailability-delays-cra-report-april-2022-final.pdf

certain period from authorisation, or any provision allowing early entry of generics in the EU market if a centrally authorised medicine is not launched in all Member States within a given number of years of granting the marketing authorisation, could have the opposite effect on developing and commercialising innovation on several Member States' publicly funded markets, reducing patient access to innovation.

Improving patient access is a joint goal and requires collaboration and commitment from all stakeholders. In that context, EFPIA and its members are working on a series of concrete proposals to improve patient access to innovative medicines and reduce inequalities across Europe. These include:

- **A commitment from the industry to file pricing and reimbursement applications in all EU countries no later than 2 years after EU market authorisation.** This commitment reflects the joint ambition of industry and society to make innovation for unmet health needs available for patients and health systems across Europe as soon as possible.

- **The creation of a portal where marketing authorisation holders (MAH) can provide timely information regarding the timing and processing of pricing and reimbursement (P&R) applications in the various EU-27 countries**, including the reasons why there is a delay in the P&R decision or why the MAH has not filed in a particular market.

- **A conceptual framework for Equity Based Tiered Pricing (EBTP)**, to ensure that ability to pay across countries is considered in the prices of innovative medicines, anchored in a principle of solidarity between countries, to reduce unavailability of new medicines and access delays.

- **Novel payment and pricing models**, when used appropriately and tailored to the situation, can accelerate patient access, allowing payers to manage clinical uncertainty, budget impact and sustainability of the healthcare system, whilst providing sufficient incentives for innovation.[4,5]

- Contributing to achieving an **efficient system of European assessments of relative efficacy at time of launch** in the context of the implementation of the Health Technology Assessment (HTA) Regulation.

The definition of EBTP is grounded in value based pricing

[4] https://efpia.eu/media/554543/novel-pricing-and-payment-models-new-solutions-to-improve-patient-access-300630.pdf

[5] https://www.efpia.eu/media/602581/principles-on-the-transparency-of-evidencefrom-novel-pricing-and-payment-models.pdf

As a concept, differential or tiered pricing has been discussed many times over the recent past.[6] Although it has been used outside of Europe, particularly, it is associated with vaccines in low and middle-income countries, its use in Europe is still debated.[7] It has been advocated by European policymakers[8] and different forms of differential pricing have been investigated by the European Commission.[9]

Building on Value Based Pricing (VBP), as the foundation for pricing innovative medicines (where the pricing medicines is based on the value they deliver to patients, healthcare systems and society), Equity Based Tiered Pricing (EBTP) is a framework for the pricing of medicines that takes into account a country's ability to pay with the objectives of improving patient access (defined broadly in terms of speed and availability) across Europe.

EBTP is a framework for ensuring that prices reflect affordability, and should be seen as building on the foundation of value based pricing (which should also ensure prices reflect the wealth of the country). The pricing of medicines based on the value they deliver to society, including the benefits they deliver for patients, caregivers, health systems and wider society, is paramount, allowing different health systems to adopt different definitions of "value" and different approaches to linking the perceived value of a certain medicine to a specific price level when making reimbursement decisions.

There is a consensus in the industry, and with other stakeholders, however that the concept of EBTP could be beneficial to all stakeholders. There is also agreement that this requires solidarity between purchasers and should be seen as only part of the solution – it will not by itself solve the problem of patient access to innovative medicines across Europe and will need to continue to evolve. However, to date there has been little specificity as to how it would work in practice.

EBTP – how it could work in practice

The commitment of both Member States and industry is needed for EBTP to work in practice, and some of the current barriers to access and differential pricing need to also be addressed. This includes application of External Reference Pricing (ERP) principles and ensuring that non-extraterritoriality is observed. EFPIA members support an EBTP approach based on a concrete conceptual framework with the following characteristics:

- To promote faster and greater access, companies would voluntarily commit to applying EBTP principles to an innovative medicine, with corresponding

[6] The terminology used in the literature on tiered pricing varies. In some cases differential pricing and tiered pricing are used interchangeably meaning that different classes of buyers are charged different prices for the same product. In some cases differential pricing is used when it is at the companies discretion how prices vary based on ability to pay, whilst tiered is used when it is a prescribed approach.

[7] It has also been used as a basis for pricing COVID related therapeutics, which are seen as a form of pilot for its wider application.

[8] https://www.ispor.org/docs/default-source/presentations/903.pdf?sfvrsn=730e5cc0_1

[9] "Study on enhanced cross-country coordination in the area of pharmaceutical product pricing: Final Report" 2015

commitments from the Commission and member states to the removal of the key barriers to EBTP.

- This requires defining rules regarding the tiers and pricing rules when EBTP is applied, while leaving room for individual company pricing:
    - A simple form of tiering: We considered a range of different tiering structures. We concluded that a simple form – two tiers based on Gross National Income (GNI) per capita adjusted for purchasing power parity (PPP) – should be included in the framework. This simple model creates an upper and a lower tier with roughly equal number of Member States placed in each (see appendix). This tiering should ensure some stability over time ensuring countries don't arbitrarily move in and out of tiers but should be open to revision reflect changing fundamentals. Companies applying EBTP could apply company specific tiering systems, which could be more sophisticated including health spending for example, or additional tiers, but must remain consistent with the industry 2-tier approach.
    - Pricing principles: There are arguments for price bands, but there was greater support for pricing principles, such as the prices in lower tier must be lower than the lowest price in the upper tier. This was sometimes described as a "best price" rule. This would ensure Member States in the lower tier pay less than Member States in the upper tier. The "best price" rule would be applied to a product's net price in the first indication at launch. The rationale being this is to improve access to innovation, rather than later in the product lifecycle. The advantage of the best price rule is that it is a clear, simple rule but it necessarily means EBTP will not be applicable to so many products.
- In order for prices to reflect value and be consistent with EBTP, companies can negotiate with individual countries in order for prices to reflect the value that medicines deliver in that market. The negotiation at member state level enables the final price to:
    - Reflect the value that a product delivers (which may vary across countries) and reflect affordability issues. Given that countries make different choices on the use of medicines in different patient populations across the care pathway, this should be accounted for in the pricing of the medicine.
    - Reflect the volume that will be used in the market. It is inevitable that markets with high volume will wish this to be taken into account in pricing.
    - Reflect the evolution of data for the product as it develops, especially for orphan, ultra-orphan, end of life and potentially curative (i.e. ATMPs) medicines where it is not possible to develop a mature data set prior to broad availability of the product, but which may demonstrate more or indeed less value as the data matures. There should be the option of price adjustment to reflect increases in value that could not be initially demonstrated, but also the option to rebate where the value demonstrated is less than initially expected.
- EBTP sets a ceiling for the tier 2 prices, but the final price is dependent on company strategy and negotiations in the Member States. EBTP does not replace value assessment or value-based pricing.

Confidentiality is a required policy to deliver patient access

The resulting price must be commercially confidential. Companies would self-identify their "best price" from the upper tier and ensure that final prices in the lower tier countries are lower than this "best price." Member States would not have visibility over what net prices would be across Europe. Though the negative impact of transparency within Europe would be mitigated by changing the ERP systems of the Member States, confidentiality would still be necessary given the use of European prices in other parts of the world. EBTP should be workable in Europe because there is overarching infrastructure and governance framework (that can address ERP) and solidarity. This is not the case globally.

Given confidentiality of prices, there will inevitably be questions as to the *credibility* of EBTP. Given the need for price confidentiality, it will not be possible to publicly observe exactly how EBTP is working in practice. A process of verification will be required. The need for a verification system causes some concern to industry participants as this is commercially sensitive information. This is mitigated if it is an independent body (separate from industry, the European Commission and payers). The body would audit a sample of products to ensure compliance. Given applying EBTP would be voluntary, this would apply only to products where the company has committed to price them according to industry EBTP principles. This will need to respect confidentiality agreements agreed with negotiating partners and would apply for the first two years after marketing authorisation (consistent with the commitment to file). There will need to be some flexibility in its application to reflect unexpected changes in exchange rates for example. This may also need to be a transition period, with flexibility in the application of rules as barriers to EBTP are removed. There will also need to be verification of Member States compliance with the application of ERP principles and ensuring that non-extraterritoriality is observed. Verification should therefore be a symmetrical requirement.

Demonstrate solidarity through the removal barriers to EBTP

For EBTP to work in practice, there needs to be Member State commitment with protections required to overcome the barriers to differential pricing in Europe. These must be based on the principle of solidarity such that any lower price agreed in relatively less wealthy countries is not used as leverage to lower prices or otherwise reduce expenditure in high income countries. Solidarity would rule out the inclusion of less wealthy countries in the ERP systems of wealthier nations,[10] and would require mechanisms to ensure that medicines sold under an EBTP framework in less wealthy markets are not unfairly diverted to wealthier markets.

What is needed is an internal market solution that mirrors the solidarity enshrined in Regulation 953/2003 to avoid trade diversion into the EU of certain key medicines delivered to developing countries under a global tiered pricing system to tackle

[10] Currently according to IQVIA analysis, there are 48 cases where a Tier 1 countries includes a much lower income Tier 2 country in their ERP rules.

HIV/AIDs and other diseases. There are a number of potential solutions available that are compliant with the competition rules and that can be deployed over time as confidence is built amongst all stakeholders to ensure that the system is workable.

A political commitment that EU Member States will adhere to the principle of solidarity and commit to good practices in ERP, including a binding commitment from Tier 1 countries not to refer to EBTP prices in Tier 2 countries would be a necessary first step. There needs to be a commitment from both Tier 1 and Tier 2 countries to adhere to non-extraterritoriality for products where EBTP is applied. In order to address diversion undermining EBTP:

- In the short-term, contractual solutions can help ensure that EBTP agreed volumes are dispensed for use within the Tier 2 Member State in question. Member States have the ability to ensure that EBTP products are dispensed to patients in their territories pursuant to any such arrangements via the European Medicines Verification System.[11]

- As soon as possible a legislative solution would provide a more solid basis to promote a broader uptake of EBTP. This could be achieved through the application of the principle of non-extraterritoriality of national price controls (as reflected in Recommendation 6 of the G10 Medicines initiative, Recommendation 9.2 of the High Level Pharmaceutical Forum[12] and subsequent EU policy documents) and as implemented in Article 94.7 of Spain's Medicines Law.

In addition, EBTP should be part of a more holistic approach to address access barriers, including adjustment of local pricing and reimbursement timelines and value assessments to conform to the 180-day timeframe foreseen in the Transparency Directive. Current delays in Tier 2 countries would materially limit the effectiveness of EBTP framework. Participation by Member States would be voluntary for a particular medicine, matching that of companies, but a process would be required to ensure participation was meaningful and predictable.

Timelines will need to be agreed and the commitments from industry should be match the commitments required from other stakeholders

EBTP delivers for all stakeholders

The initial testing undertaken by the industry has shown that EBTP delivers to **all stakeholders:**

[11] Data stored in the National Medicines Verification Systems includes real time granular data regarding the number of packs supplied by manufacturers (including the number of packs dispensed in national pharmacies, number of packs exported (and/or imported), as well as stock levels in the supply chain. For further information see also 'A proposal for Using the Data Repositories for Shortages Monitoring', Bouvy & Rotaru, Frontiers in Medicine, January 2021

[12] https://op.europa.eu/en/publication-detail/-/publication/4fddf639-47cc-4f90-9964-142757d2515a

- **Patients:** The fundamental objective is to improve patient access to innovative medicines in less wealthy member states. Reduced delays mean patients across Europe can get faster access to life-saving medicines.
- **Member States:** Public payers across all Member States can be satisfied they are getting a price that reflects both the product's value and a country's ability to pay. This means less wealthy member states will have a guarantee that they will receive lower prices relative to richer member states and their patients get access to medicines more quickly.
- **European Commission and European Parliament:** Reducing access delays in less wealthy Member States is consistent with the European Commission's objective of reducing health inequalities across the EU and ensuring all member states have better access to healthcare and medicines.
- **Industry:** EBTP could be a positive proposal to address unavailability and delays. Faster access ultimately means industry is able to generate revenue from their products earlier. This earlier generation of revenue in less wealthy Member States can help compensate for the lower prices provided in these markets.

The EBTP conceptual framework outlined in the present discussion document relies upon reciprocity of commitments among stakeholders. **The proposed industry commitments laid out in this document would therefore be contingent on the implementation of corresponding commitments from other parties necessary for the EBTP framework to achieve the intended impact**.

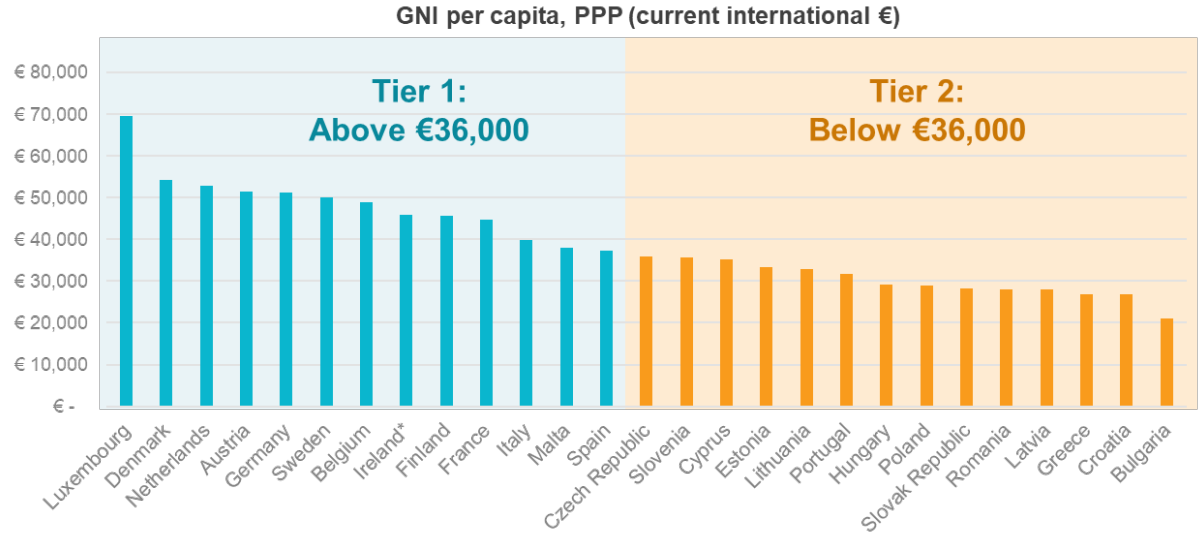
March 2022

Appendix: How might EBTP work in practice

Approach to tiering

A simple approach to tiering is necessary, and this should be determined by wealth and ability-to-pay only. Gross National Income (GNI) per capita PPP is an appropriate indicator as this ensures pricing reflects a country's ability to pay for treatment, while allowing for cost differences between countries. This is also a commonly used indicator; the European Commission largely uses GNI to determine Member State payments to the EU budget (where their contribution is determined by applying a percentage to each Member State's GNI). Figure 1 below illustrates what a two-tiered approach could look like:

Figure 1: Approach to EBTP with two tiers based GNI per capita PPP



*Note: *Modified Gross National Income (GNI) is an indicator designed specifically to measure the size of the Irish economy by excluding Globalisation effects.*

Pricing principles

EBTP pricing principles that companies would need to follow to apply EBTP:

- Companies will need to agree to application of industry tiers and pricing approach
- The price in individual markets should be determined by negotiation.
- A sample of net prices across products should be provided to an independent audit process upon request, with justification for prices varying beyond the factors included EBTP.

Process for applying EBTP