efpia
European Federation of Pharmaceutical Industries and Associations

ADDRESSING PATIENT ACCESS INEQUALITIES IN EUROPE

The Industry commitment to file pricing and reimbursement applications across Europe and the European Access Portal

MARCH 2022

THE RATIONALE FOR PROACTIVE INDUSTRY PROPOSALS

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. These are shown in Figure 1 below.

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.

Figure 1: The root causes of delays and unavailability

CATEGORY	POTENTIAL ROOT CAUSES
The time prior to market authorisation	• The speed of the regulatory process • Accessibility of medicines prior to marketing authorisation
The price and reimbursement process	• Initiation of the process • The speed of the national timelines and adherence
The value assessment process	• Misalignment on evidence requirement • Misalignment on value and price • The value assigned to product differentiation and choice
Health system readiness	• Insufficient budget to implement decisions • Diagnosis, supporting infrastructure and relevance to patients
Delay from national to regional approval	• Multiple layers of decision-making processes

Source: The root causes of unavailability and delay to innovative medicines

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

There will continue to be calls for greater transparency regarding these underlying root causes, as has already been seen in the EU Pharmaceutical Strategy (see Box 1).

Box 1: Discussion of root causes in the EU Pharmaceutical Strategy

> Innovative and promising therapies do not always reach the patient, so patients in the EU still have different levels of access to medicines. Companies are not obliged to market a medicine in all EU countries; they may decide not to market their medicines in, or withdraw them from, one or more countries. This can be due to various factors, such as national pricing and reimbursement policies, size of the population, the organisation of health systems and national administrative procedures resulting in smaller and less wealthy markets in particular facing these problems.

Source: European Commission, EU Pharmaceutical Strategy[4]

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 in the context of its 2021 open public consultation on the revision of the general pharmaceutical legislation. These proposals include stepping up co-operation with and among Member States on the affordability of medicines as well as potential introduction of 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. In most countries, the inclusion of the product on the reimbursement list will determine availability and access. Any requirement for MAHs to place a centrally authorised medicine on the market in the majority of Member States (including small markets) within a 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, significantly reducing patient access to innovation.

Improving patient access is a joint goal and requires collaboration and commitment from all stakeholders. EFPIA and its members have worked on a series of concrete proposals to improve patient access to innovative medicines and reduce inequalities across Europe. These include inter alia:

- **A commitment from the industry to file pricing and reimbursement applications in all EU countries no later than 2 years after EU market authorisation, provided that local systems allow it.** 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 European Access 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. The focus of this document.
- **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.[5,6]
- 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.

This document focuses on the European Access Portal. It aims to outline the vision behind this initiative, its aims, and how it can improve patient access to innovative medicines across Europe.

4 - Communication from the Commission to the European Parliament, the Council, the European Economic and Social Committee and The Committee of the Regions, Pharmaceutical Strategy for Europe COM/2020/761 final

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

6 - www.efpia.eu/media/602581/principles-on-the-transparency-of-evidencefrom-novel-pricing-and-payment-models.pdf

THE ROLE OF A EUROPEAN ACCESS PORTAL IN IMPROVING MEDICINES AVAILABILITY

The industry shares the goal to make its innovative medicines available and accessible to all patients in the EU that need them. It commits to file Pricing and Reimbursement (P&R) applications for EU authorised medicines in all EU-27 Member States within two years after the grant of the EU Marketing Authorisation, provided that national pricing and reimbursement systems allow. As part of this commitment, it will set up a Portal where MAH will be requested to provide timely information regarding the timing and processing of 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 for P&R in a particular market.

The role of the Portal is to improve transparency regarding the root causes of unavailability and delay, including the role of the environment. It would add new information to the debate on the filing for pricing and reimbursement and the reasons why we see no application for reimbursement or a delayed process allowing the root causes of unavailability to be recognised. This will allow data on delay and lack of availability to be put into context and support the broader understanding that it is a shared responsibility, requiring a shared solution.

This new set of evidence reflects industry's willingness to meet societal concerns and should provide a sound basis for a structured dialogue with relevant stakeholders at a pan-European level. This objective would be to address issues around availability, accessibility and affordability, including through exploring models to make medicines prices better reflect both the value they deliver for patients and societies and the economic level of countries for a more equitable and sustainable system. The industry calls upon Member States, with European Commission support, to progress concrete solutions to evidence-based access hurdles, including amending External Reference Pricing systems towards improved patient access in all EU countries and increased solidarity between Member States' healthcare systems.

The Portal, which will be operated by IQVIA on behalf of industry stakeholders including EFPIA, will be formally released in April 2022. The information in the portal will increase the transparency around market launches and access delays for new innovative medicines across countries, and help identify and address hurdles, by:

- Timely collection and publication of the considerations underlying unavailability and the degree to which this reflects (a) barriers within the environment, and (b) commercial decisions arising in light of Member States' pricing and reimbursement processes. This will facilitate deeper understanding of the root causes of delay and unavailability of innovative medicines in Europe and therefore enable the healthcare community to better work together to address the barriers to access identified.
- Regular updating of this data would enable tracking of progress towards improving patient access to innovative medicines and reducing delays and disparities.
- By assembling information enabling benchmarking of the timing of marketing authorization approvals in Europe and other world regions (e.g. China, Japan, UK, US), the Portal could prove to be a valuable tool to monitor the relative attractiveness of Europe's regulatory framework, and to measure the global impact of potentially significant foreign policy developments on access to innovation in European markets.

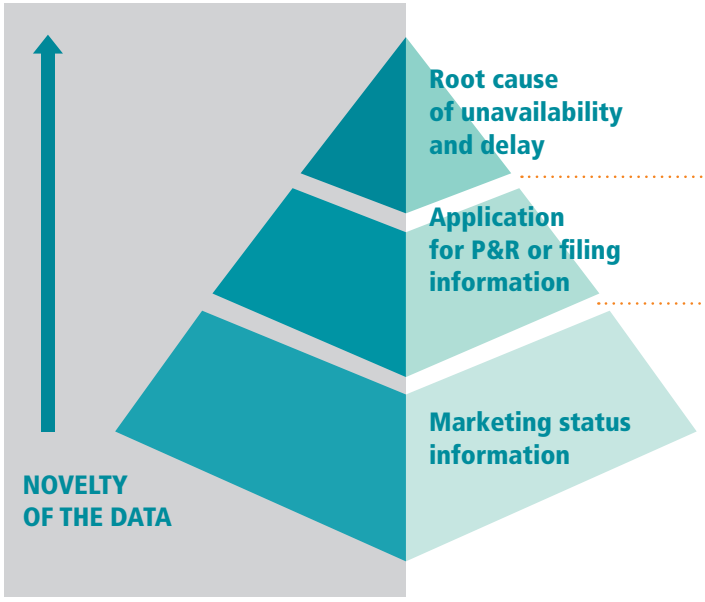
THE ADDED VALUE OF A PORTAL FOCUSED ON TARGETING EUROPEAN ACCESS HURDLES

There are long-established initiatives which collect data on the marketing status of medicines in Europe. The Portal builds on these by providing information on the timing and outcomes of the pricing and reimbursement process, and the reasons behind these.

The Portal brings the missing piece to the existing WAIT database and existing initiatives aiming at providing greater transparency relating to availability of centrally approved products on the EU markets by providing evidence on:

- Filing for price and reimbursement (including timing of filing);
- A timely qualitative assessment of the main reasons for unavailability across markets and the length of the delay;
- The link between data on unavailability and delay and the root causes and how this changes over time.

Figure 2: Potential for more granular data on unavailability and delay



- The reason why a company has not filed for P&R in a particular market
- The reason why there is a delay in the decision of Pricing and Reimbursement
- Ex-post information to be collected through EFPIA's Portal Targeting European Access
- EFPIA's WAIT has information on availability and delay
- IRIS portal collects information on marketing status for al CAPs
- Ex-ante information collected on planned availability through DG Sante's pilot

HOW WOULD AN EFPIA PORTAL TARGETING EUROPEAN ACCESS HURDLES WORK IN PRACTICE?

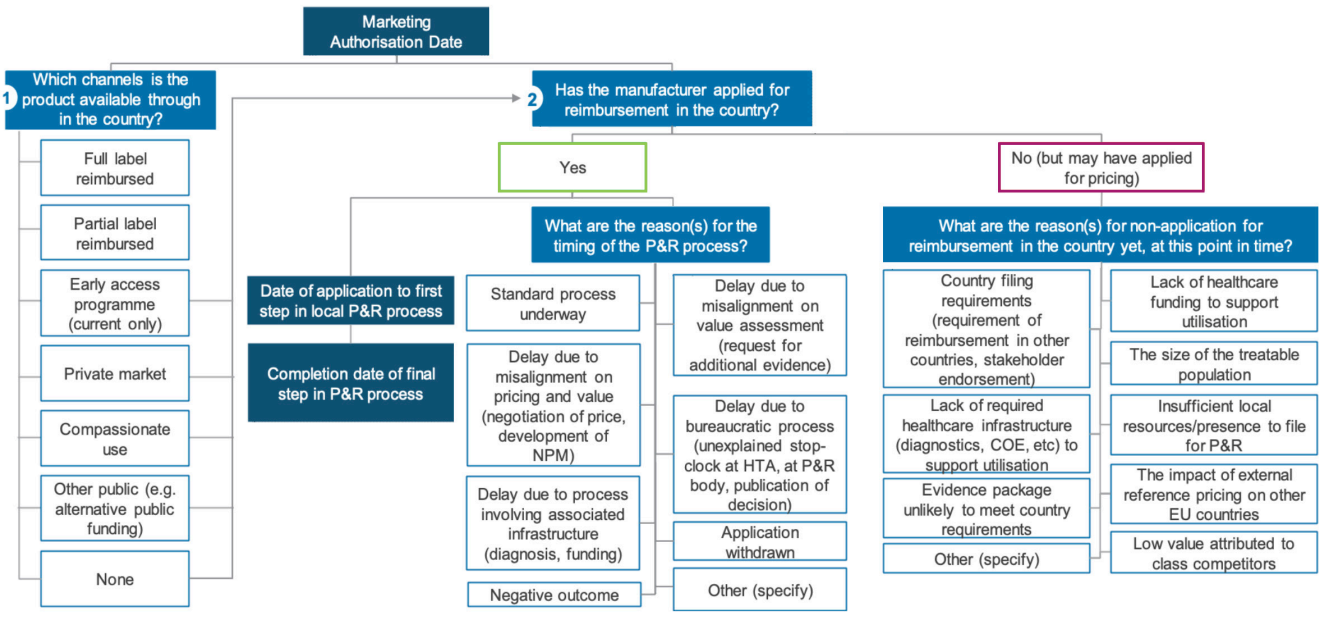
The Portal is an online interface where medicines manufacturers can input information about the pricing and reimbursement status of their medicines in each country in the EU/EEA, on a voluntary basis. The Portal was developed according to the following guiding principles:

- The database should include only ex post data, to limit the extent to which this is commercially sensitive (data on the timing of a pricing and/or reimbursement application should not be considered as commercially sensitive when disclosed ex-post within a certain timeframe);
- Any new portal should work closely with existing databases, to ensure there is no duplication and administrative costs are minimised. Some of this could be pre-filled from public sources (drawing on published regulatory data, data submitted to IRIS and WAIT);
- A company's own information (individual product related information) would only be publicly disclosed if that company agrees to it.

THE INFORMATION COLLECTED IN THE PORTAL

As described above, the aim of the portal is to collect information on the timing and outcomes of pricing and reimbursement processes for recently approved products in Europe, and the reasons behind this, reflecting the full 10 root causes set out in the EFPIA/CRA paper. To this end, the Portal will collect the following information about each product in scope, in each Member State and at a particular point in time, as illustrated in Figure 3 below.

Figure 3: Schematic of information collected through the Portal



MAH will be able to select multiple options to indicate all the applicable answers for each product in each Member State. This will provide a comprehensive picture of the barriers within the access environment of each Member State, and how this impacts the decision-making of MAH.

THE SCOPE OF THE PORTAL

Products eligible for inclusion in the Portal will be innovative medicines or biosimilars (which have different issues affecting unavailability) which have been granted marketing authorisation in their first indication in Europe from January 2021 for a four year period. This time period has been selected to ensure a focus on recently approved medicines so that the data collected in the Portal reflects the current pricing and reimbursement environment in Europe, while allowing a sufficiently large time interval so as to collect a meaningful sample of medicines.[7] It is important to acknowledge when reviewing and using the data from the Portal that the access challenges for the first indication may differ from challenges to later indication.

The objective of the Portal is that it will include data from the widest range of manufacturers, including smaller companies with different access challenges. Although the submission of data into the portal will be on a voluntary basis, we would expect a strong industry participation as the Portal will be used a Flagship for those companies showing their commitment to addressing access related hurdles by providing more transparency on the challenges they face on the various EU markets.

It is important to note that the focus of the Portal will be on the "availability" component of access to medicines. As outlined in the paper "The root cause of unavailability and delay to innovative medicines", patient access to medicines is dependent on three milestones being achieved: granting of a European marketing authorisation, inclusion of the medicine in a national reimbursement or insurance scheme[8] and post-availability access processes such as regional or hospital negotiations, to enable the medicine to reach the right patients[9]. The Portal focuses on the reasons behind unavailability or delay related to the second of these three milestones, that is the national reimbursement process in each Member State. The Portal aims to qualitatively examine the root causes related to availability in this context and not provide a quantitative picture of patient access in each Member State (as is provided through WAIT).

VALIDATION OF THE PORTAL

IQVIA will set up an 'appropriate usage governance board' composed of a representative of each industry stakeholder association and other interested stakeholders in order to:

- **Set rules** for data usage including approved standard use case: anonymised benchmark reports for manufacturers; anonymised report for countries as a diagnostic tool; list of involved companies;
- **Review data** use on a periodic basis;
- **Provide reassurances** to new organisations that information within the portal is being collected and stored appropriately in a way that that supports new entrants.

PUBLICATION OF INFORMATION COLLECTED IN THE PORTAL

A key topic is how information collected through this initiative will be disclosed. At this stage the disclosure of information at the aggregate or therapeutic level is likely to be appropriate. Greater transparency could be considered at a later stage but companies should be free to decide to disclose product-level data at any time. The report would be updated every six months, to ensure it continues to reflect an up-to-date snapshot of the European access hurdles.

Publication will allow a picture to be built up of what is causing the unavailability of medicines in Europe and disparities across countries. The whole healthcare community will then be better equipped to work together to address key hurdles and realise the great opportunity that new medicines provide to transform patient's lives, health systems and wider society.

7 - Existing products with approval of new indications will not be included, consistent with the WAIT and IRIS databases.

8 - In some cases, products may be available without reimbursement on the private market, but this does not ensure wide access to the patient population.

9 - https://www.efpia.eu/media/554527/root-causes-unavailability-delay-cra-final-300620.pdf

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