



European Alliance for
Personalised Medicine

Affordable and sustainable patient access to personalised medicine

1. The promise of personalised medicine

Personalized medicine, or PM, promises to revolutionize healthcare, with its key goal of providing the right treatment to the right patient at the right time. It holds the potential to substantially improve patient care and patient outcomes while supporting efforts aiming at more efficient healthcare systems.

PM has the potential to optimize the delivery and dosing of treatments so patients can receive the most benefit at the least amount of risk and harm, eliminating both the unnecessary side effects of toxic treatments such as chemotherapy and the delays associated with the “trial-and-error” process that many patients endure to obtain the correct diagnosis and treatment for their condition. And when personalized therapies prove more effective or present fewer side effects, patients may be more likely to adhere to their treatments for longer. PM, which is at the cutting edge of innovation, has started to feed into better health outcomes and a more efficient utilization of scarce healthcare resources.

PM uses diagnostic tools to identify specific biological markers, often genetic, that can help assess which medical treatments and procedures will work best for each patient. By combining this information with an individual’s medical history and circumstances, PM allows doctors and patients to develop targeted prevention and treatment plans.

On the one hand, managing biomarker research and development efforts adds extra complexity to PM research and development programs. At the same time, PM is expected to become a key driver of more efficient clinical development programs which are by far the most expensive element of medicine R&D (approximately 40-60% of the investment). Through more relevant pre-selection of patients, a trial would be populated with a much better defined population with a higher likelihood of a better therapy response resulting in faster results and an overall lower failure rate of trials. This can eventually result in smaller and quicker clinical trials from which patients would benefit through earlier access to the medicine.

These advances have already impacted the way patients are treated. Metastatic melanoma and certain types of lung cancer are now further classified by their molecular signatures, and can be treated with the drug that is most likely to improve the patient’s chance of survival based on that signature. The number of available PM products has grown substantially in recent years. These innovations are changing the face of health care today, as researchers further investigate the heterogeneity of disease and work together to develop solutions that improve patient care and support a more efficient utilization of healthcare resources.

At the same time, patient access to innovative PM technology and services remains highly suboptimal and varies dramatically between EU Member States. The fact that a new medicine or innovative product can sometimes take as long as 20 years or more to get from bench to bedside is not only clearly undesirable but is arguably unacceptable in the 21st century. And even after all those years of translation, patient access in the absence of flexible pricing and reimbursement systems is frequently restricted to patient subgroups and often further delayed or not affordable at all in less affluent parts of Europe.

If the potential of PM is to be realized, changes will be necessary in the way medicines are developed, regulated, assessed and rewarded. It is necessary to make policymakers and payers realize that investing now in these advanced therapies and technologies for PM as well as in adequate regulatory and payer decision making frameworks will be a key pre-requisite to see their potential long-term, cost-effective patient outcome benefits and more efficient health care systems materialize.

The proposed solutions range from better coordination and collaboration models between stakeholders and decision makers at various stages within the bench-to-bedside timeframe and across countries to more sophisticated pricing, reimbursement and funding mechanisms as well as effective forms of utilization management to address the inherent complexity of co-dependent PM technologies and services.

2. Assessing and optimizing the value proposition of PM technology and services: the role of HTA

In most European countries formal assessments of health technologies and services (HTA) are used to inform pricing, reimbursement, funding, and/or utilization decisions. It is used to support (and not to pre-empt or even to replace) evidence-based resource allocation decisions taken by health care payers and policy makers throughout the lifecycle of a technology.

HTA can help decision-makers with the evidence-based selection of health technologies and services and the equally important task to improve their effectiveness and efficiency over time. Increasingly, payers are severely restricting or even deny patient access to health technologies and services when there is no clear or exceptional value as they seek to cut costs and to increase the efficiency of the healthcare system. HTA acts as a supportive process that allows payers to seriously consider whether or not to fund one treatment over another and to make it available to those patient groups that will be most likely to benefit from it.

HTA is described as a multidisciplinary process that summarizes in a systematic, transparent, unbiased, and scientifically robust manner information about the medical, social, economic, organizational, legal and ethical issues related to the use of a health technology in the specific context of a national health care system. Its aim is to inform the formulation of safe and effective health policies that are patient focused and seek to achieve best value. Despite its policy goals, HTA is expected to always be firmly rooted in research and the scientific method.

Arguably, the public is one of the most important stakeholders in decisions related to PM, yet there is a paucity of studies regarding citizens' values, concerns and expectations towards PM. Further efforts to deliberate with the public are warranted to inform the effective, efficient and equitable translation of PM into routine practice. As the science, delivery and organization of PM develops, understanding the public's perceptions, values and expectations is important given that the public is not only its main beneficiary but also its primary funder. Without developing an understanding of citizens' concerns and expectations, HTA and decision makers may miss major drivers and barriers for the successful integration of PM into routine care. Unfortunately, current HTA is rarely concerned with assessing the wider societal benefits and risks of health technologies and services. Without recognizing these wider aspects of the societal value proposition of PM, suboptimal incentivization of corresponding innovation activity is a likely consequence.

Although the economic aspects of a health technology or service are considered to be just one of the key criteria of HTA, and although many HTA agencies emphasize the relevance of criteria other than cost-effectiveness, it remains large unclear how important the various HTA criteria are relative to one another, and how they are taken into account in recommendations and subsequent decisions. There is a considerable need to increase the transparency about the criteria upon which health care resource allocation decision are made. Current decision making processes are rather opaque, leading to a lack of public confidence in the decisions that result.

Multi-criteria decision analysis is a methodology that could be used to support deliberative processes of healthcare decision makers by structuring, segregating and providing transparent access to evidence (incorporating quality assessment), while facilitating communication about value judgments and data needs among stakeholders.

Recommendation(s):

Generating evidence on citizens' expectations and the wider societal benefits and costs of PM technology and services is a pre-requisite for their successful introduction into routine care. Actively back the adoption of support methodology for HTA, appraisal and patient access decision making that helps to ensure that multiple and conflicting decision criteria are fully recognized and dealt with in a transparent and balanced manner.

Although HTA and patient access decision making cannot be done without a thorough understanding of the potential or actual value of a health technology or service within the specific real life conditions of the national health care system, very little is done to actively solicit perspectives of patients and their healthcare providers whose behavior will have the biggest impact on the efficient and effective utilization of these technologies and services.

Patients have valuable perspectives and experience that can inform HTA and decision making, helping to explain what it is like to live with a condition, experience with current technologies and what they would most value in a new treatment. These perspectives can inform trial designs that include outcomes of importance to patients and that do not put onerous burdens on the patients in a trial thus improving compliance and maximizing data collection. In the appraisal phase of HTA, patient evidence can inform discussions about the added value of a new technology and generally inform the value judgments of researchers and decision-making committees. The active involvement of patients will result in technology assessments of higher quality that are more widely accepted and stand a greater chance of being implemented.

Recommendation(s):

Patients are at the center of the health care process and should be more actively involved in HTA and appraisal

Existing recommendations and methodology to incorporate patients' perspectives in HTAs should be further advanced and promoted.¹

Member States with limited experience of involving the patient and citizen perspectives in HTA should be supported through best practice sharing.

Leverage existing HTA capabilities and capacity through improved EU-level collaboration

Substantial efforts are underway at both, national and EU-level, that aim to realize efficiencies in the conduct of HTA. EU-level actions with the focus on a common assessment of the relative (clinical) effectiveness of a technology can add value if they avoid unnecessary duplication as part of HTA in individual Member States and enable greater clarity, lead to an improvement in standards of methodological and process aspects in HTA, improve predictability, and contribute to better and timely access of health technologies to patients.

Progress in this area requires a stronger commitment by payers to integrate EU-level HTA findings into their national decision making processes in order to materialize the expected efficiency gains from avoiding duplications of effort. While smaller Member States as well as those that have not yet build a larger capacity to conduct HTA express considerable interest in EU-level HTA collaboration, other Member States find it considerably more difficult to integrate the results of EU-level HTA activity into national decision making processes.

There are considerable differences of how HTA is used in the various Member States as well as in their underlying methodological frameworks. In some countries HTA is only used to support price negotiations in other countries HTA also guides technology utilization and decisions to limit patient access to subgroups with perceived higher average outcome benefits. Some countries employ formal cost-utility analysis e.g. in the UK, formally integrating clinical and economic assessments by calculating the cost of interventions against the health benefit gained (mostly expressed in quality adjusted life-years - QALY), and deciding on coverage by the payer/healthcare system according to whether its cost/QALY in relevant patient subgroups is below or above a more or less formal threshold. Others do not conduct formal economic ("efficiency") assessments but focus on a comprehensive clinical assessment of the added clinical benefit of a new technology (e.g. Germany).

Given all these differences in how HTA is used in national decision making, collaborative European efforts are best suited at the level of the clinical aspects of HTA. Context-specific aspects of HTA like

¹ Health Technology Assessment International (HTAi), a global scientific professional society in the field of HTA, has established quality standards for the involvement of patients (and citizens) in HTA which were developed via an extensive, rigorous, international research process. The HTAi standards assume that patient involvement can happen throughout the HTA process from scoping to dissemination and are particularly relevant when HTA is used in decision making about reimbursement or access. They may be of particular use to HTA organizations to help them review their own patient involvement processes. [<http://www.htai.org/index.php?id=744>]

efficiency considerations (“economic evaluation” and/or price negotiations must remain at national level to reflect potentially different healthcare priorities, recognize differences in affordability, and the availability of infrastructure that is required to efficiently implement PM.

Recommendation(s):

Understand the limits of the current HTA approaches for PM and develop mechanisms to establish internationally shared standard for relative effectiveness assessments in general and for PM technology and services in particular.

Ensure that technology assessments that were jointly conducted by national HTA agencies can be accepted as input in patient access decision processes in the respective countries.

Adapting HTA to the new paradigm of PM

For PM it is vital to ensure that HTA will be able to support timely patient access decisions for a set of co-dependent technologies e.g. a diagnostic kit and a therapy. Patient access to co-dependent PM technology and their potential health outcomes will inevitably be compromised when healthcare providers will not be able to utilize the diagnostic services that are required to identify a PM’s target population in a highly accurate and reliable manner. The consequences of misleading diagnostic information may include the use of an ineffective therapy or the consequences from other suboptimal, and sometimes irreversible, medical decisions. HTA processes must be able to adequately address the co-dependency of PM technology and to reflect it in specific recommendations. This is currently not the case in many countries which makes it difficult for the diagnostic industry to understand its business case for investments in this area.

Many HTA bodies generally prefer to base technology assessments on randomized controlled trials (RCTs), but these are not always available for PM technology and services. While the call for prospective RCTs may make sense in some cases, it is, when applied without distinction, hindering progress in the field of PM.

There needs to be a flexible approach to the generation of clinical utility data for diagnostic tests, which balances the need for high-quality evidence with incentivizing innovation. The rapid pace at which technology changes for the diagnostics sector means that a strict insistence on RCTs, for instance, may render a new diagnostic outmoded by the time pre-market evidence is generated. Different approaches to evidence generation will need to be considered by HTA bodies and payers as well as regulators, including real-world data and observational studies. It is also important that the pricing and reimbursement system takes account of the cost of evidence generation in appraising the value of the diagnostic. For example, a double randomization model for the development of combination stratified medicine and diagnostic should not become a requirement.

Recommendation(s)

HTA processes must be able to adequately address the co-dependency of PM technology and to reflect it in specific recommendations.

Where adopted by HTA agencies, “absolute” hierarchies of evidence should be replaced by accepting diversity of approaches in reflection of the level of evidence that can reasonably be expected given the specifics of a technology or service.

Additional research should be encouraged to develop and improve evidentiary and analytical methodology and standards that fit the specifics of PM and the needs of decision makers for reliable and generalizable evidence.

HTA agencies as active participant of a continuous stakeholder dialogue about evidence generation

HTA has traditionally been considered as an activity taking place before launch once the regulatory marketing authorization process is completed. On one hand this is too early because at the time of launch it will usually not have been possible to learn about the healthcare system’s challenges to effectively and efficiently use the new technology or service i.e. the primary focus of relative effectiveness research and HTA. On the other hand, only deploying HTA resources at the time of launch comes too late when technology developers - in the absence of early dialogue opportunities with HTA agencies – have missed the opportunity to establish relevant evidence for later HTA processes. Frequently, it will not be possible to conduct the studies required to close these evidence

gaps once the technology is launched. This may have considerable downstream consequences for patient access.

Increased dialogue early in development and along the life-cycle of products, involving all decision-makers and the manufacturer, could help identify the right level of requirements and reduce duplication between different bodies. It would also help the acceptance of different types of data by different decision-makers.

There is a major difference between Member States about when HTA is used. Some use HTA at the time of launch to inform the first pricing, reimbursement and related patient access decisions ('rapid' HTA), which is based on information collected in clinical trials conducted pre-launch, and HTA later down the line ('full' HTA or re-assessments), which can integrate more information on the actual utilization and outcomes in routine care.

The long-standing tendency to one-off decision-making at the time of marketing authorisation is starting to give way to more dynamic, progressive approaches which are based on an understanding how the value of a technology evolves over time. In this context HTA may be most valuable if it acted as a "change agent" and to explore how health care providers and other stakeholders can be incentivized for a more active collaboration towards improved efficiency i.e. reduced costs and/or improved outcomes over time ("dynamic approach"). At the time of launch of a health technology or service, HTA should be able to provide an early prognosis of its value and to identify potential levers to dynamically influence its utilization to become more effective and efficient over time. This may be more important than to act as mere "gatekeeper" tasked with a comprehensive assessment of its potential effectiveness and efficiency before it is even launched ("static approach").

Recommendation(s):

Ensure that capacity of HTA bodies is allocated to support their engagement in the early and continuous dialogue with technology developers about evidentiary expectations for new technologies and services, involving other decision makers, stakeholders, and experts as required. Strengthen the capability and re-allocate capacity of HTA bodies to assess existing, potentially obsolete health technologies and services in order to update guidance for their optimized utilization.

3. Incentivizing affordable PM innovation: pricing, reimbursement, and funding decisions

Regardless of the ever-growing body of research and the game-changing technologies that will make PM technically feasible, it will be the ability and willingness of governments, payers and industry to invest into these new technologies that will determine whether patients will have access to these innovative treatments. The willingness of healthcare payers, and subsequently that of providers and innovative healthcare companies to invest into PM technology and services will largely depend on the type and level of incentives that societies put in place to encourage innovative efforts. Health care policy makers and payers need to respond to the key role they play in incentivising and providing access to innovation by actively evolving their HTA, pricing and reimbursement systems to become more transparent and specific about what constitutes value and how it will be rewarded.

The success of PM – the value created by directing treatment to responders, and away from non-responders – depends upon the clinical utility of the diagnostic test in combination with the PM as demonstrated by evidence and ultimately the quality of available diagnostic services when implemented under routine care conditions. Lack of evidence risks unnecessary harm to patients through inappropriate clinical decision-making based on an inaccurate diagnosis. However, several factors act as a disincentive for the generation of robust evidence in diagnostic development: lack of effective intellectual property protection, competition from less strictly regulated laboratory- or in-house developed tests, and the lack of requirements or rewards for evidence generation under the current regulatory and pricing and reimbursement systems. Pricing and reimbursement systems must consider the costs of evidence generation and not simply the costs of production.

Recommendation(s):

A new and broader definition of value, which explicitly includes the full benefits of PM, needs to be developed and integrated into HTA, pricing and reimbursement systems for drugs and diagnostics.

Pricing and reimbursement systems must reward and incentivise the continuous development of diagnostic technology and services as well as the respective evidence for their improved analytical, clinical performance, and clinical utility.

Pricing and reimbursement systems for PM technology should be able to (a) enable prices adjustments over time to reflect increases and decreases in value, and (b) to manage two diagnostic scenarios: companion tests of one biomarker and large platform tests of multiple biomarkers.

To incentivise the generation of evidence about analytical and clinical performance and clinical utility successfully, consideration should be given to the promotion of commercially approved diagnostic tests unless an 'in-house' test has evidence of equivalent or improved quality. The use of tests without performance evidence needs to be controlled, using a combined approach of regulation and clinical guidance.

Coordinating assessment and reimbursement mechanisms for drugs and diagnostics

Traditionally health care systems have implemented separate patient access pathways for drugs and diagnostics which are based on fundamentally different principles. While many countries have introduced some form of value-based pricing for pharmaceuticals, reimbursed prices for testing services are traditionally not based on their specific value but on a cost-plus model. As a consequence, prices for companion diagnostics insufficiently reflect their value in accurately and reliably selecting the right patients for a specific therapy. This leads to considerable variation in patient access to the most accurate and reliable companion diagnostic technology within and across EU Member States with severe implications for the appropriate utilization of the corresponding PM. Patients who have been falsely classified as responders will be treated with a medicine that is a suboptimal treatment choice in the best case, but may cause serious harm in the worst case.

In a number of EU countries companion diagnostics are not funded by the healthcare system. In countries without clear funding pathways for companion diagnostics patients do not have access to PM unless the manufacturer of the pharmaceutical funds the companion diagnostic.

The major obstacle to patient access appears to be inconsistent funding decisions. Different decision makers typically are responsible for drug and companion diagnostic reimbursement and funding decisions. In many countries decisions about the reimbursement of companion diagnostics are not only separated from those for medicines but also further delegated to individual fundholding hospitals. The consequence are inconsistent patient access conditions for drugs and companion diagnostics with the potential highly variable patient treatment outcomes and inequitable access to state-of-the-art PM technology and services.

The current model of one drug paired with one companion diagnostic is likely to change in the near future with platform diagnostics that will direct the use of different drugs for multiple indications. A system will need to be developed to evaluate the value of these platform diagnostics.

Recommendation(s):

Funding and reimbursement decisions for co-dependent PM and companion diagnostic should be closely coordinated and take place at the same level in the health care system

Advance the existing HTA, pricing and reimbursement framework to adequately assess, appraise and incentivize appropriate utilization of emerging diagnostic approaches that identify multiple biomarkers using a single panel test (such as a series of specific gene mutations), thereby challenging the idealised drug–diagnostic co-development model. This will increasingly be the case with advances in next-generation sequencing technologies.

Post-launch value assessments: addressing uncertainty where it matters most

Facing rising expectations from patient and health care providers together with uncertainty on both costs and effectiveness, payers are forced to explore new methods and techniques for clinical and financial risk management when allocating resources to promising health technologies and services.

PM with its inherent promise to reduce uncertainty about the intended therapeutic outcomes, directly responds to major requests that payers have with the introduction of new health technologies and services:

- ┌ Increased certainty about diagnosis and mechanism of disease

- ┌ Improved estimation of patients' risks of later outcomes (e.g., prognosis), which could influence treatment management decisions
- ┌ Better prediction of response to therapy or drug metabolism rates or a reduced potential for adverse events
- ┌ Reduced wastage of health resources associated with treating nonresponders
- ┌ Improvement in the quality and cost-effectiveness of patient-tailored treatment versus empirical approaches to prescribing

PM can only deliver against its potential to effectively reduce outcome uncertainty if it is optimally utilized e.g. if the right patients are identified. Reducing “utilization uncertainty” may be of particular relevance when a medicine is priced differently between indications. This may be the case when the value of this medicine is perceived differently between indications.

More and more, innovative approaches to manage the entry of new medicines, commonly referred to as managed entry agreements (MEAs), are used in a number of EU Member States. A MEA can be defined as an arrangement between a manufacturer and payer/provider that enables reimbursement of a medicine subject to specified conditions.

Although combinations are possible, there are two main groups of MEAs: one group links pricing and reimbursement to health outcomes (e.g. payment for performance, coverage with evidence development); the second facilitates access to new medicines through the use of financial instruments which are typically linked to the actual utilization of a technology or service (e.g. price-volume agreements, rebates).

The development of these MEAs reflects the fact that essential evidence for the value of a technology or service in routine care can only be obtained post-launch. Collection of this information allows tailoring HTA recommendations on the basis of real-life utilization, effectiveness and other outcomes data in different patient subgroups rather than solely based on evidence from randomized controlled clinical trials (RCT)-derived efficacy.

The use of patient registries would support the decision-making process, inform clinical practice, and could provide information about long-term adverse events. Given the associated costs of registries it is crucial to set up patient registries in a flexible way to collect sufficient data and to account for the evolution in patient population and treatment strategies. At present, many registries are collecting data only on a specific technology or service which is do the fact that their manufacturers are the only source of funding. More sustainable disease- rather than technology-focused registries will only be established if the current lack of public funding for data collection initiatives can be overcome. Given the limited number of patients in certain countries there seems to be considerable added value for cooperation at EU level that could lead to larger and efficient data collection. Given considerable level of investment that is required to establish and main patient registries it will be essential to identify and involve all stakeholders that can benefit from the collected data.

Recommendation(s):

Uncertainty that is present at the time of launch of PM technology and services should be systematically addressed when this is cost-effective, timely and realistic to gather evidence that helps inform future decision making.

Regulatory, HTA and other stakeholder needs for evidence post-launch should be coordinated to make efficient use of limited resources.

Efficient data collection infrastructure should be established to monitor utilization and/or effectiveness of medicines when used under routine care conditions at national level which can be linked at EU-level.

All stakeholders should continue to focus evidence development on pre-launch clinical development programmes due to ethical, methodological, and other limitations of post-launch evidence generation.

P&R conditions should be regularly assessed, in order to adapt to new data produced post-launch; MEAs are an essential element in a longer-term evolution of adaptive patient access pathways, i.e. adaptive licensing and an accompanying framework of flexible pricing and reimbursement mechanisms.

Prices that reflect a different value in different indications

Classifying diseases at the genetic, molecular and cell functional levels will introduce a stepwise change in disease treatment and prevention that is already far advanced for some diseases. For example, the stratified treatment of a number of cancer types has delivered substantially improved health outcomes. Cancer encompasses a complex group of diseases traditionally defined by where in the body it originates, as in breast cancer or lung cancer. This framework for studying and treating cancer has made sense for a long time, but molecular analysis now shows that cancers of different organs have many shared features, while cancers from the same organ or tissue are often quite distinct. For example, some types of gastric cancer look very similar to certain breast cancers, and recognizing those similarities may lead to the development of new medicines. The identification of biomarkers that can be used across a range of tumor types to indicate which therapies effective in one cancer type could be of value in others have already led to the development of PMs that can be used alone or in combination to improve patient outcomes. The use of specific PM approaches either alone or in combination with others across multiple indications will emerge increasingly complex treatment pathways.

Currently, pricing mechanisms are not aligned with these scientific advances nor the current economic situation. New and flexible approaches, including multi-indication pricing are required in order to avoid the unproductive interference of rigid pricing and reimbursement mechanisms with scientifically meaningful research and development initiatives. Flexible pricing and reimbursement systems have the potential to better reflect the value of individual medicines that are supposed to be used in different approved indications and treatment combinations so as to ensure patients have access to the best medicines both now and in the future. Multi-indication pricing is already used in a number of European countries, such as Italy where it is most advanced.

Flexible pricing approaches like multi-indication pricing work through the collation and analysis of highly secure, anonymised, aggregated patient data from real world medicines utilization. Data registries will provide a broad value to the system beyond the implementation of flexible pricing models: from policy decisions and budget management to research activity and appropriate utilization and improved clinical performance, the wider potential to truly contribute to an optimal healthcare future is significant.

Recommendation(s):

Actively explore the practical feasibility and acceptability of differential pricing for different indications of the same medicine in order to incentivise the optimal development and utilization of PM technology and services.

Making innovative PM technology more accessible in the European Union

Today there is a substantial inequality in patient access to medicines in Europe, be it between countries or between regions in countries. This is not compatible with European social values and policies for cohesion. Keeping in mind the requisites on equity and (international) solidarity, a crucial challenge within the EU context is to make valuable health care innovation accessible to all EU citizens which requires solidarity within Member States and solidarity between Member States. Ideally, valuable PM technology should be accessible to all patients that could benefit from them at an affordable price – without diminishing incentives for future innovation.

Adapting prices of pharmaceuticals to reflect the ability to pay in different countries can offer a win-win situation in terms of generating dynamic efficiencies that contribute to patient access to PM technology, the sustainability of healthcare systems and the pace of innovation. This is highly relevant in the European context where the gaps between GDP and healthcare spend per capita and access to the latest innovative medicines have been widening and are significant. Yet today's pricing and reimbursement practices have the effect of discouraging price differentiation. Member State action and support from the EU are required to create a framework to enable and encourage voluntary recourse to differentiated pricing.

Differential pricing would mean that pharmaceutical companies are able to offer medicines at prices that better reflect the economic situation of different countries. This is currently discouraged by formal and informal international price referencing (IRP) practices whereby national authorities determine the amount to which they will reimburse a drug by looking at what other countries are paying – using a basket of other countries as a benchmark to set drug prices in their own country.

International price referencing (IRP) mechanisms can be especially problematic in tough economic times. The pharmaceutical industry is often called on to assist poorer countries with cost savings by cutting prices: Recognizing the need to support patient access to medicines, the industry has accommodated weaker economies accordingly. However, if those exceptionally low prices are adopted via IRP mechanisms by richer economies then the whole business model is challenged. In practice IRP leads to a leveling of prices in higher- and lower-income countries therefore undermining equitable and affordable patient access among EU citizens. The industry needs to be able to help out where it's necessary, without fear that revenues in richer economies will collapse in a way that undermines its ability to invest in the medicines of the future.

Recommendation(s):

Member States to take reasonable measures to facilitate the introduction of effectively differentiated pricing policies that reflect variations in ability to pay at national level

While IRP is inherently problematic as a means of ensuring optimal prices, these negative consequences could be at least reduced if international reference pricing systems were operated according to an established set of principles e.g. requirement to only reference economically comparable countries e.g. with a comparable GDP per capita.

Member States should take the necessary steps to ensure that medicines specifically priced for patient groups who would not otherwise be able to afford them are delivered to those patients and are not otherwise diverted

Key references

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