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Fair Pricing of Innovative Medicines: An EHA Position Paper

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The problem

High prices keep innovative medicines out of reach for many patients across Europe, resulting in growing inequalities in accessibility and standards of care. Budgetary pressures compel payers and insurers to make increasingly difficult choices, at the expense of patients and investment in innovation. Costly new therapies become available only for the lucky few —or for no one in those countries that lack the purchasing power or are left out of manufacturers' marketing strategies altogether. With expensive combination therapies adding to the problem, the potential of scientific and medical innovation remains underused, not least in hematology. By severely limiting the uptake of novel gene and cell therapies, high prices are undercutting efforts to increase, personalize and optimize treatment options for patients suffering from blood disorders.[1]

Causes

High prices result from a complex set of interrelated factors. Causes can roughly be divided into three categories:

Business models

Manufacturers are primarily driven by the need to recoup the costs of research and development—of products that eventually make it onto the market and of those that don't—and by a desire to offer substantial return on investment to investors. In a system that rewards patent rights with market exclusivity and which obscures R&D costs, price-setting and profit levels, bringing a profitable drug to market continues to be the main incentive, not the patient and public interest.

Market access

The authorization of drugs for the European market is based exclusively on criteria of safety and efficacy. As long as these are not supplemented by affordability, added-value and quality-of-life criteria, profit-driven development will prevail over models that put the patient and public interest first.

Pricing and reimbursement

Decision-making by public payers and insurers is done at the national level, based on different models and methodologies, rather than at the European level in a harmonized, concerted manner. Similarly, price negotiations with pharmaceutical companies are conducted by national governments and in secrecy, instead of collectively and transparently. This fragmentation has negative consequences for overall price levels, (widely disparate) accessibility for patients, value for (taxpayers') money and the sustainability of health systems.

The challenge

The overall picture is one of imbalance between public and private interests all along the research-to-market development chain.[2] Too often, rewarding (commercial) innovation and investors prevails over the need to ensure access and affordability for patients and health systems.

Clearly, achieving a perfect balance between private and public benefits is not an end in itself. Nor is the reduction of prices per se. The common goal of all stakeholders, in our view, must be to develop and agree on a set of principles and practices for ***fair pricing***, that is, pricing that offers a proper and socially acceptable reward for developers and funders of innovation while ensuring ***affordable access to the best possible treatments for all patients across Europe***.

Solutions

In light of the sizable challenges and the disparities across countries, no single, one-size-fits-all solution is possible. A number of elements can be identified however that will have to be part of any model for ensuring fair pricing and affordable access to innovative medicines:

Intergovernmental collaboration on procurement and reimbursement

Although more a response to, than a solution for the fundamental defects of existing pharmaceutical business models, cooperation between national governments on the procurement and reimbursement of the growing number of very expensive medicines is a necessary first step. The *Beneluxa Initiative on Pharmaceutical Policy*[3] offers the best example of how such cooperation can strengthen payers' negotiation power in the face of the secretive divide-and-conquer approach traditionally adopted by pharmaceutical companies. Beneluxa has explored joint price negotiations and, potentially more impactful, joint horizon scanning. Its creation of the International Horizon Scanning Initiative (IHSI) addresses the information asymmetry between governments and companies and establishes an 'early-warning system,' allowing payers to better anticipate the arrival of expensive new medicines. By exchanging information and coordinating their 'willingness to pay,' the participating countries effectively form a united front that can make their collective red lines, rather than the company's demands, the starting point of price negotiations.

Collaborative, harmonized *Health Technology Assessment (HTA)* would be another meaningful step towards ensuring access to the best possible medical care at the best possible price. Joint EU-level clinical assessments as proposed by the European Commission[4] will increase efficiency and reduce cost by pooling resources and expertise, streamlining regulatory processes and providing uniform guidance for Member States' pricing and reimbursement decisions.

New economic models

While increased cooperation is essential, more fundamental change is needed. The case for developing a new economic model for pharmaceuticals—one that is transparent, balances the interests of all stakeholders and puts patients at the center—has been made by EHA previously.[5]

In our view, the following principles should underlie any new model for the development, marketing, pricing and reimbursement of pharmaceuticals:

a) The whole decision-making chain from drug development to pricing and reimbursement should be patient-centric, rather than drug-centric. Patients must be (meaningfully) involved throughout.
b) New medicines entering the European market should *not only* be safe and efficacious (the criteria for authorization by the European Medicines Agency, EMA); they must *also* be affordable and accessible for patients and public health systems.
c) In addition to safety, efficacy, affordability and accessibility, relevant clinical and patient benefit should be a key factor in pricing and reimbursement decisions. No public resources should be wasted on medicines that offer little or no added value compared to treatments already available. It is therefore important to enhance the ability of health systems "to review and adjust prices, and to withdraw funding for superseded or less cost-effective medicines if required," as suggested in a WHO report on the pricing of cancer medicines.[6] The importance, as well as the complexity and the challenging nature of increasing the value of pharmaceutical spending has been aptly described by the OECD.[7]
d) Any form of pricing coordination at the European level will have to take into account the differences in purchasing power between countries. A 'one size fits all' solution will not be possible.
e) Transparency is a pre-condition for developing any effective and broadly accepted approach to 'fair pricing.' Manufacturers invoking costs to justify extraordinarily high pricing of their products cannot leave patients and (tax)payers in the dark about investment and profit levels. More openness from industry on bench-to-market costs needs to be matched by transparency from payers on the cost-benefit considerations underlying pricing and reimbursement decisions. An interesting model has been proposed by the International Association of Mutual Benefit Societies (AIM), in which a fixed amount of R&D costs for each new drug is used as the basis for calculating fair prices. If a pharmaceutical company wants to charge a higher price, it will have to provide full transparency on cost.[8]

In an ideal scenario, the existing EU framework for market approval would be complemented by a mechanism for joint HTA (EU-level clinical assessments) as well as structural EU-level coordination on pricing. Along the lines of the model proposed by Uyl and Löwenberg,[9] such a mechanism would combine respect for national price-setting competencies with a collective, multi-stakeholder process for determining an upper limit to the price of a new innovative drug. All collectively determined maximum prices will have to be transparent, realistic and fair. Pricing deliberations will have to take into account the clinical-scientific evaluation of a treatment, the pharmaceutical industry's data and interests, and affordability for patients and health systems.

With innovative drugs becoming ever more costly, societal willingness to pay for health gains, relative to disease burden and budgetary impact, becomes an important factor—see Annemans's commendable concept of 'VIA pricing' (value-informed, affordable prices for innovative medicines).[10]

Thus, in addition to safety, efficacy and cost-effectiveness, socially and economically acceptable pricing must be a pre-condition for reimbursement.

Incentivize affordable, accessible, sustainable innovation

Since the causes of high prices are diverse and complex, no single measure or model will suffice to push pricing levels down. Intensified collaboration at the EU/international level and the

introduction of new economic models will have to be supplemented by a balanced set of incentives to keep innovation affordable and accessible for patients and health systems.

Active encouragement—which can take the shape of financial incentives, regulation or awareness campaigns—is needed in particular to:

- spur innovations in the diagnosis and treatment of **rare diseases** (see the EHA position paper on affordable access to orphan medicinal products[11]).
- ensure affordable and equitable access to **novel cell and gene therapies**, such as CAR T-cell therapy. These innovative treatments represent a major step forward in personalized medicine, however the high cost of developing and administering them, combined with the lack of a harmonized European approach to clinical trials, regulatory approvals, HTA and reimbursement, has slowed down their uptake and is keeping them out of reach of the majority of European patients.
- increase access to **publicly funded, cheaper clinical trials** that are investigator-driven,patient-centered, risk-based, and less bureaucratic. In addition, development of practice-oriented 'real-world trials' may contribute to the optimization and personalization of treatments, and thus to ensuring that scarce healthcare budgets are spent on therapies that are cost-effective and of real added value to patients.
- boost the uptake of **biosimilars**, which are as safe and efficacious as their reference products but tend to be considerably less expensive. By helping to drive down the prices of the reference biologics themselves, as well as across product classes, the overall pricing impact of biosimilars is significant and likely to increase.[12,13]
- establish public-private partnerships that deliver affordable innovation, respond to unmet clinical needs and ensure that the interests of both public and private investors are served.

We would also incentivize, rather than enforce, **transparency** on costs and price-setting. While we are a strong proponent of transparency, it should not be imposed as a stand-alone measure. Forced openness on pricing and cost could actually have a reverse effect on access and affordability, especially in middle- and lower-income countries, as Chalkidou et al have argued in response to the WHO report on cancer pricing.[14] The dilemmas around price transparency, as articulated by the OECD,[15] seem impossible to resolve without a fundamental overhaul and EU-wide harmonization of how pricing and reimbursement decisions are made.

Conclusion

High prices are a serious impediment to the uptake of innovative treatments and the sustainability of health systems. They are by no means the only factor influencing availability and accessibility, and it is important to stress that we do not regard the reduction of high medicine prices as an end in itself. Affordability is however crucial for improving access to the best possible treatment for patients across Europe, which must be the primary goal for healthcare professionals, the pharmaceutical industry and public health systems.

The problem of (excessively) high prices has many causes and can only be solved through a combination of targeted policies, regulatory measures and collaboration between stakeholders. A holistic approach is needed, which takes the whole product lifecycle into account from development to uptake—with the patient interest at the center at all times.

While much can be done at the national and regional level, achieving real and lasting results is only possible through collaboration at the European level. An EU policy and regulatory framework for HTA, pricing and reimbursement is needed. Where that is not (yet) possible, voluntary collaboration and coordination between EU Member States needs to be strongly encouraged. The EU and national authorities must heed the calls for collaborative action from patients, healthcare professionals and payers to ensure affordable and equal access to innovative medicines across Europe.

Key messages for policy makers and stakeholders

Affordability is crucial for improving access to innovative treatments for patients across Europe, and must be the primary goal for healthcare professionals, the pharmaceutical industry and public health systems. Fair pricing solutions must be developed by and with all stakeholders, in a way that benefits both public and private stakeholders, and with the patient's interest at the center, at all times.

EHA calls on **EU Member States** to step up collaboration and coordination on HTA, horizon scanning, price negotiations and reimbursement.

EHA calls on the **EU institutions** to develop a pharmaceutical strategy that:

- enables and supports collaboration between national authorities on pricing and reimbursement with a dedicated EU policy and regulatory framework
- prioritizes structural frameworks for harmonized HTA and coordination on pricing
- takes a holistic lifecycle approach, supplementing the safety and efficacy criteria at the heart of EU market authorization with affordability, accessibility, cost-effectiveness and (clinical/patient) benefit as requirements for reimbursement
- offers a balanced set of incentives to keep innovation affordable and accessible for patients and health systems
- promotes a new economic model for the development, marketing, pricing and reimbursement of pharmaceuticals that puts the patient at the center, balances the interests of public and private stakeholders and takes into account the differences in purchasing power between countries (no one-size-fits-all).

EHA calls on **all stakeholders** to develop and agree on a set of principles and practices for fair pricing, ie, pricing that offers a proper and socially acceptable reward for developers and funders of innovation while ensuring affordable access to the best possible treatments for all patients across Europe.

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