EXECUTIVE SUMMARY:

THE VALUE AND PRICING OF INNOVATIVE MEDICINES

EPF European Patients Forum

APRIL 2020

This is a summary of the **European Patients' Forum's** position paper on the **Value and Pricing of Innovative Medicines**, developed in consultation with our membership and published in June 2019. The paper contributes EPF's perspective as a cross-disease umbrella patient organisation to the EU-level and international debate on prices and value of innovative medicines, building on the core principles of 2016. For a more detailed understanding of EPF's views, please refer to the full position paper[1].

All patients in the EU have a right to access high-quality, patient-centred care in a timely manner. This not only improves patients' health and well-being, but offsets significant costs to the health and social systems as a result of avoidable exacerbation of health conditions. Medicines form one of the most important aspects of treatment for many patients. However, there are concerns in Europe and globally that the cost of some new medicines is undermining health systems' capacity to provide sustainable and equitable access for all.

Inequalities in access to medicines are contrary to the EU Charter of Fundamental Rights, the EU Treaties' commitment to the principle of well-being and to the fundamental European values of equity, solidarity and good quality in healthcare. Patients will only benefit from new, innovative therapies if they are available in a timely manner, accessible and affordable to all who need them. EPF considers an "innovative" medicine to be not only new, but something that brings real and concrete added value for patients over and above what exists already.

Medicines should be available at a price that is fair, reasonable and sustainable

Recalling the principles of availability, accessibility, affordability, appropriateness and adequacy[2], EPF believes decision-making on the pricing of new medicines needs to reflect several factors. The added value of a new medicines for patients should be a strong consideration when considering what is a fair and sustainable price. In addition, other factors must be taken into account to ensure medicines are accessible, including the costs of research and development; direct and indirect contributions from public funding; affordability to patients; and impact on national health budgets.

Debate with all stakeholders, including organisations representing patients and informal carers, is needed around adequate and sustainable investment in health, defining valuable innovation, societal values and preferences, and what constitutes a fair price or acceptable return on investment for industry and for society.

The pharmaceutical system should move to greater transparency whilst guarding against unintended negative impacts on access

Transparency is essential for the accountability of the system towards patients and citizens, to correct asymmetries of information between payers and industry, and to generate trust. Transparency should help develop regional collaborations among Member States on pricing and reinforce Member States' negotiation capacity, especially small ones. However, more knowledge is needed on possible unintentional consequences of full price transparency of actual negotiated prices in the long-term.

Differential pricing has potential to improve access in low-income countries, and for that reason should be further explored as a political strategy. Possible consequences of increasing transparency on the feasibility for differentiating prices should be taken into account, if it might affect patient access. Member States should act on the basis of solidarity and fairness, ensuring that poorer Member States get a fair deal, and should act to limit the negative impacts of parallel trade where necessary, on grounds of public health.

In addition, the processes and criteria of decision-making along the medicines pathway from marketing authorisation through to health technology assessment, pricing and reimbursement must become more transparent and understandable to patients and citizens. Patients' perspective should be meaningfully embedded at each step, with patient representatives involved in decision-making.

Patients' perspectives are crucial for accurate assessment of the value of innovation

EPF understands an innovative medicine to be a new medicine that brings real and concrete added value for patients, over what exists already. The determination of added value is only possible with the involvement of patients, yet patients' views are still insufficiently accounted for. Many clinical trials still do not include measures for outcomes that matter to patients, including quality of life, and patients are not involved in a systematic and meaningful manner in health technology assessment.

Industry and academic researchers should ensure that meaningful patient involvement is embedded in the R&D process, so that new medicines can demonstrably present added value for patients. Similarly, HTA bodies should ensure that patients are fully included in the HTA process, in line with EPF's recommendations[3]. Patients' views should be taken into account in discussions around pricing and reimbursement decision-making. Finally, a more comprehensive, robust and smart approach for collecting real-world data, with full respect of patients' rights, privacy and confidentiality, is needed, and mechanisms for patient input must be expanded and strengthened, both at EU level and nationally.

Public investment in health, including health promotion, prevention, good quality care, and research, are political choices. EPF calls on policymakers to take the necessary action to progress towards an inclusive society that values health and recognises its vital contribution to growth and social cohesion[4]. EPF also calls on the pharmaceutical industry to embed patients' priorities in their research and development activities in a way that is meaningful, non-tokenistic and respectful of ethical guidelines. Companies should commit to, and act on, greater transparency and follow good commercial practices, and ensure the products they develop provide added value for patients and are priced reasonably so they are affordable.

1. Link to the online paper when published - 2. EPF (2016) "Defining and Measuring Access to Healthcare: the Patients' Perspective". Position paper available at www.eu-patient.eu/globalassets/policy/access/epf_position_defining_and_measuring_access_010316.pdf - 3. See EPF's position paper on HTA and joint statement of patient organisations, available at http://www.eu-patient.eu/News/News/patient-organisations-have-co-signed-a-joint-statement-on-hta/ - 4. EPF (2017) "Taking Action – A Roadmap to Achieving Universal Health Coverage for All by 2030" available at http://www.eu-patient.eu/globalassets/campaign-on-access/taking-action---a-roadmap-to-achieving-universal-health-coverage-for-all-by-2030.pdf

EPF CORE PRINCIPLES AND RECOMMENDATIONS

EPF calls on policymakers and industry to apply the following Core Principles and Recommendations:

Health and access to innovative medicines

1. Health is a fundamental right *as well as* a critical investment in the well-being, economic development and cohesiveness of society.
2. Medicines are not a consumer good like any other; and patients' lives cannot be measured in purely economic terms. Medicines are an essential public good and a core element of health policy.
3. Patients' needs go beyond medicines and include other therapeutic options, social and community services and peer support. Innovation should be encouraged in this wider sense, encompassing better ways of structuring and delivering integrated health and social care; more efficiency and effectiveness; social innovation; and the development and effective use of new user-driven technologies.

The centrality of patients

4. A common understanding is needed on the concepts of "innovation", "value" and "added therapeutic value". Patients' views should be central to this understanding, including patients' perceptions of quality of life, patient-relevant clinical and quality-of-life endpoints, and patients' views on benefit/risk.
5. Patients should be recognised as an essential stakeholder group in medicines pricing and value assessment, and the patient perspective should be at the heart of every assessment.
6. Investment is needed in frameworks, structures and methodologies for meaningfully incorporating patients at all stages, from setting research priorities to clinical research, regulatory assessment, Health Technology Assessments, and pricing and reimbursement decisions.

Call for action to EU Member States, European Commission and Pharmaceutical Industry

1. The European Commission should implement the European Parliament's call to set up a ***High Level Strategic Dialogue*** co-ordinated by the Commission, which should build on the achievements of the High-Level pharmaceutical Forum and include patient organisations, to reflect and establish concrete and comprehensive strategies to achieve a framework for fair and equitable access in the short, medium and long term.
2. A ***framework for fair and equitable access*** should maximise societal benefit and patient access whilst avoiding unacceptable impact on healthcare budgets should be developed at EU level, through a consultative process led by governments with the participation of all stakeholders including patients. Such a framework should encompass at least the following elements:
   - Closer collaboration by Member States on price negotiations and scaling-up of pilots on early dialogues;
   - Transparency of real prices, at least to Member States and other payers in their negotiations with industry;
   - Adoption of common principles and mechanisms for encouraging and rewarding innovation in order to encourage continued investment in R&D, based on the evaluation of the current EU IP and incentives legal framework;
   - Exploration of innovative models for incentivising research & development especially in areas of high unmet need;
   - Exploration of the potential of optimal use of mechanisms such as adaptive pathways, managed entry agreements and others for optimising access and determination of value;
   - More thorough exploration of differential pricing mechanisms, barriers and potential solutions to dealing with practical issues such as parallel trade;
   - Common EU principles for calculating a fair price, taking into account the specifics of each Member State.

3. ***Pricing and reimbursement authorities*** should be transparent about their decisions, how these are made, what criteria are used, and who is involved in the process. Information explaining decisions should be available in an easily accessible and understandable format that addresses the specific questions of patients and the public.
4. ***Cooperation between Member States*** on medicines pricing should take place on the basis of cross-EU solidarity and include meaningful involvement of patient organisations as well as an appropriate level of transparency towards patients and the public.
5. ***The real costs of developing the therapy and/or acquisition*** must be made transparent, including contributions from public investments, infrastructure, etc.
6. ***Pharmaceutical companies*** should price new medicines fairly and responsibly to ensure that they are accessible and affordable. Pricing should consider inter alia a country's relative capacity to pay; budget impact; the extent of public funding that contributed to the development of a medicine; and the need to ensure universal access.
7. ***The European Commission should collect and analyse data and provide public reports on access to medicines and access barriers faced by patients*** in different EU member states, including medicine shortages, bad commercial practices and price increases including of "repurposed" products, and other barriers.
8. The ***EU should foster research and incentives based on patients' unmet needs*** including under-represented patients (such as women, older people, children). Adequate EU investment in ***biomedical research*** should be secured in the future 9th Framework Programme, and funding for patient organisations' involvement in research projects should be ensured.
9. EU public funding for research should focus on patients' unmet needs, and should ***build in a return on the public investment*** with conditions such as affordable and equitable access, non-exclusive licencing and open access publication of results. Open data requirements should be strengthened and incentivised.
10. ***Transparency of the entire system*** must be improved, including transparency of research, registration and publication of all clinical trials, and transparency of financial and other links between the industry and public institutions, healthcare professionals, academic researchers and non-governmental organisations.

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