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EAHP Position Paper on Access to Medicines

Meeting the needs of patients!

EAHP's Position Paper on Access to Medicines advocates for affordable medicines of good quality that are provided in a timely manner to patients. To achieve this goal barriers to treatment access need to be broken down and the uptake of enablers that promote and safeguard the access of patients to both new life-saving medicines and older, essential medicines must be increased.

Barriers to treatment access

- Lack of purposeful procurement practices
- National pricing and reimbursement policy choices jeopardising patients' adequate access
- Medicine shortages
- Unavailability in certain markets, leading to inequity between Member States.

Enablers to treatment access

- Health Technology Assessments (HTAs), including common reports at EU level
- Collaboration and best practice sharing on pricing and reimbursement
- Increasing the use of prevention measures Fostering innovation and research

To achieve an equilibrium between the barriers and the enablers to treatment access EAHP:

- recommends that the expertise of the hospital pharmacist in pharmacoeconomics and the assessment of drug effectiveness be leveraged and well utilised within value-based evaluation approaches. Additionally, the implementation of the forthcoming HTA Regulation should be used for the expansion of healthcare professional input in HTAs at both European and national level.
- supports the view of EURIPID and strongly recommends that this tool is not applied on its own but in conjunction with other policy measures, including transparency.
- calls on hospital managers and its members to work together to increase the uptake of risk assessments in hospitals.
- urges increased investment to support the development of innovative proposals and the encouragement of practice-based research projects to investigate new fields of infectious disease control such as immunotherapy and to optimise the cost-effectiveness of systems for surveillance on antibiotic use and resistance.

In striving for a European Health Union aided by the implementation of the Pharmaceutical Strategy, EAHP is committed to working together with the European institutions and other stakeholders by giving a voice to access issues that otherwise might be forgotten.

Hospital pharmacists across the world are working every day for their patients to ensure that they receive the medication they need to improve their health and to prevent and cure diseases. However, sometimes the medicine that is suited for an individual patient is not accessible. Growing healthcare expenditure has become a problem for many European countries. Innovative drugs, in particular, place an additional strain on already tight hospital budgets. Patients are directly affected and increasingly faced with avoidable accessibility and affordability issues. Besides the constraints faced by public health budgets, there are other

barriers to treatment access. These include the growing problem of medicines shortages[1], delayed market access for new treatments in some European regions[2] or increased out of pocket costs for patients.[3]

Hospital pharmacists have a direct stake in the efficient functioning of the medicines supply chain and the operation of medicines reimbursement systems that enable patients in hospitals to benefit from sustainable and equitable access to the medicines they require. To ensure that patients' needs are met, the position paper of the European Association of Hospital Pharmacists (EAHP) on access to medicines explores the barriers to treatment and possible enablers that provide patients with timely access to safe, effective and affordable medicines.

Barriers to treatment access

Since the global financial crisis in 2008, public healthcare expenditure has come under greater scrutiny. This trend has slowly started to reverse again with growth rates for healthcare spending increasing.[4] Although medicines pricing and reimbursement is a national competence, the challenges posed by rising costs for new medicinal products have moved this topic into the limelight at the European level. In its 2016 Conclusions, the Council of the European Union highlighted the problems that innovative medicinal products present to individual patients and public health systems alike.[5] In March 2017, the European Parliament called for national and EU-wide measures to guarantee the right of patients to universal, affordable, effective, safe and timely access to essential and innovative therapies.[6] Since then different movements, like the Beneluxa initiative and the Valetta Declaration, have formed bringing together countries interested in jointly tackling the issue of unsustainable prices, by aiming at improving patient access to innovative medicine, cost containment and pushing for improved price transparency. Not only Member States, but also hospital pharmacists are concerned by the rising prices of medicines used in the hospital sector, since this trend in some cases, jeopardises patients' adequate access to essential pharmaceutical products. **National policy choices, linked to procurement, value assessments and pricing mechanisms should consequently safeguard the access of patients to both new life-saving medicines and older key medicines needed for their treatment**.

However, not only the costs of the product but also its **unavailability in certain markets** create barriers to treatment access. With its 'Europe's Beating Cancer Plan' the European Union has set out to revolutionise cancer prevention, treatment and care since in the EU alone 2.1 million people were diagnosed in 2020.[7] With cancer figures growing, also the development of new and innovative treatment options is rising. These novel treatments are however not reaching all patients equally. Even after receiving an EU-wide marketing authorisation, some companies are opting for only releasing their products on certain markets. Cancer care is just one example that illustrates how delayed market access can have detrimental effects on the health of patients in need of novel treatment options.

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[1] European Association of Hospital Pharmacists. 2019 Medicines Shortages Report – Medicines Shortages in European Hospitals. Brussels: European Association of Hospital Pharmacists (EAHP), 2019. Available from: https://www.eahp.eu/sites/default/files/eahp_2019_medicines_shortages_report.pdf.

[2] Uyl-de Groot CA, Heine R, Krol M, Verweij J. Unequal Access to Newly Registered Cancer Drugs Leads to Potential Loss of Life-Years in Europe. Cancers (Basel). 2020 Aug 17;12(8):2313. doi: 10.3390/cancers12082313. PMID: 32824444; PMCID: PMC7464890.

[3] Álvarez-Gálvez J, Jaime-Castillo AM. The impact of social expenditure on health inequalities in Europe. Soc Sci Med. 2018;200:9-18. doi:10.1016/j.socscimed.2018.01.006.

[4] OECD/European Union (2020), Health at a Glance: Europe 2020: State of Health in the EU Cycle, OECD Publishing, Paris, https://doi.org/10.1787/82129230-en.

[5] Council conclusions on strengthening the balance in the pharmaceutical systems in the EU and its Member States, OJ C 269, 23.07.2016, p. 31. Available at: https://publications.europa.eu/en/publication-detail/-/publication/b49097b2-5096-11e6-89bd-01aa75ed71a1/language-en.

[6] European Parliament resolution of 2 March 2017 on EU options for improving access to medicines (2016/2057(INI)). Available at: http://www.europarl.europa.eu/sides/getDoc.do?type=TA&language=EN&reference=P8-TA-2017-0061.

[7] COM(2021) 44 final. Communication from the Commission to the European Parliament and the Council. Europe's Beating Cancer Plan. Available from: https://eur-lex.europa.eu/legal-content/en/TXT/?uri=COM%3A2021%3A44%3AFIN.

Another key issue for the access to medicines is the problem of **medicine shortages** which in accordance with research carried out by EAHP has been growing over the past years.[8] Medicines shortages are not only associated with negative patient impact but they also pose clinical and financial burdens and create additional work for hospital pharmacists who are tasked with sourcing alternatives or finding other solutions to overcome the shortage. Patients are directly affected through delays in their care, suboptimal treatment options or termination of care. The 'Pharmaceutical Strategy for Europe' has recognised the problems that are caused to patients by the lack of access to medicines triggered for example by medicines shortages. However, concrete proposals for preventative action or mitigation measures are still scarce.

Procurement policies are a tool that can help with containing certain access barriers, but only if applied correctly. Negotiations that are driven mainly by pricing parameters often provide major short-term costs savings while having a potential inherent risk of negative consequences such as medicine shortages and a long-term rise of prices in a 'winner takes it all' scenario. Producers do not always have the manufacturing capacity to meet the potential need and vulnerability to the supply chain is added if alternative suppliers drop out of the market due to loss of tender. Thus, while on the one hand having the capability to enable better treatment access procurement policies can on the other hand also be perceived as a treatment barrier, especially when their application leads to market concentration and dependency on one single supplier.

Enablers to treatment access

To overcome some of the accessibility barriers to care both national and European policymakers are looking at different tools which could help with improving access of patients to the care they need. With more and more new treatment options being developed for certain patient groups, health technology assessments (HTAs) have become indispensable for measuring the added value of new medicinal products, medical equipment, diagnostic and treatment methods, rehabilitation, and prevention methods against existing ones. Hospital pharmacists, like all other healthcare professionals, have the ethical duty to ensure that patients are provided with access to the most appropriate treatment, and especially to the medicines and medical devices essential for improving their health. Previous initiatives that foster cooperation in the field of HTA such as the European Network for Health Technology Assessment (EUnetHTA) and the Adopting Hospital-Based Health Technology Assessment (AdHopHTA) brought significant benefits through sharing best practices developed by individual Member States and hospitals. Their achievements include reducing duplication of work, supporting less experienced countries and assisting with addressing health inequalities between countries, therefore ensuring equal access for patients. Hospital pharmacists await the forthcoming adoption of the HTA Regulation with anticipation since it is expected to provide the framework to further leverage these achievements in the best interest of European patients and healthcare systems. They support collaboration among Member States but have also noticed that the requirement for healthcare professional input and engagement in HTAs is low. For prudent and well-informed public policy, **EAHP recommends that the expertise of the hospital pharmacist in pharmacoeconomics and the assessment of drug effectiveness be leveraged and well utilised within value-based evaluation approaches. Additionally, the implementation of the forthcoming HTA Regulation should be used for the expansion of healthcare professional input in HTAs at both European and national level.** Similarly, their expertise should also be used in relation to the introduction of more biosimilars to the hospital formulary, which can enable access to innovative therapies for a much broader number of patients at an early stage.

The use of some form of external reference pricing (ERP) to inform decisions on prices of new and innovative pharmaceutical products is increasingly normal in the European context.[9] The EURIPID Collaboration, a voluntary and strictly non-profit cooperation between mostly European countries on

[8] Miljković N, Batista A, Polidori P, et al. Results of EAHP's 2019 Medicines Shortages Survey. European Journal of Hospital Pharmacy 2020;27:202-208. Miljković N, Gibbons N, Batista A, et al. Results of EAHP's 2018 Survey on Medicines Shortages. European Journal of Hospital Pharmacy 2019;26:60-65.

[9] Habl C, Schneider P, Németh G and Šebesta R, Euripid Guidance Document on External Reference Pricing (ERP). Final Version 8.1 of 31 July 2018. Available from: https://ec.europa.eu/research/participants/documents/downloadPublic?documentIds=080166e5bca29a81&appId=PPGMS.

building up and maintaining a database with information on national prices and pricing regulations of medicinal products in a standardized format, is one example. As outlined in the EURIPID Guidance Document on ERP, this tool should not be used as a stand-alone policy option but rather in a mix with other instruments also taking into account the principles and recommendations presented by EURIPID. **EAHP supports the view of EURIPID and strongly recommends that this tool is not applied on its own but in conjunction with other policy measures, including transparency.**

After years of spearheading the awareness-raising discussion about the influence that medicines shortages have on both patient care and the work of hospital pharmacists, the problem has gained recognition and efforts have finally shifted towards finding preventative and mitigating actions that could help with keeping the growing negative impact of medicines shortages at bay. Prospective risk assessments are one tool with which healthcare institutions could better predict emerging risks from shortages and prevent them from occurring.[10] However, despite the potential of prospective risk assessments, their uptake is slow, in particular since data, knowledge and skills on how to apply risk assessment in everyday practice are lacking. **Based on the need for more preventive measures and the benefits that prospective risk assessment present, EAHP calls on hospital managers and its members to work together to increase the uptake of risk assessments in hospitals.** For EAHP it is of uttermost importance that the proposals for the diversification of the supply chain, the adoption of preventive and mitigating measures as well as the improved notification of shortages to healthcare professionals and patients are implemented promptly in collaboration with all stakeholders. EAHP and its members are committed to continue their work linked to medicines shortages and support European action on this issue.

Another tool to improve treatment access, in particular for unmet medical needs, is **research**. For rare disease and paediatric patients research has an important societal value because it can help improve their quality of life and increase highly limited treatment options. The current rewards and incentives included in the legislation for both orphan and paediatrics medicines are generous but imbalanced.[11] Thus, leading to only supporting developments for profitable diseases or keeping products from reaching all markets across the EU even after years since the initial marketing authorisation has been issued. The revision of the legislative framework for children and rare diseases needs to factor in these shortcomings to balance the incentives for developers and the needs of patients.

However, not only research into rare and paediatrics disease is needed but also approaches that stimulate the development of new antibiotics are lacking. Despite the commitments of the European Commission included in the One Health Action Plan in relation to the support of research and developments not much progress has been made. **EAHP urges increased investment to support the development of innovative proposals and the encouragement of practice-based research projects to investigate new fields of infectious disease control such as immunotherapy and to optimise the cost-effectiveness of systems for surveillance on antibiotic use and resistance.** These recommendations should be factored into the flagship initiatives related to antimicrobial resistance that are being commenced as part of the implementation of the 'Pharmaceutical Strategy for Europe'.

When it comes to generating data and evidence through clinical trials, EAHP recognises that not all patients are suitable candidates for them. However, efforts should be made – taking into account also all relevant constraints – to create clinical trials that also study the effects of new treatment options in paediatric populations, pregnant and breastfeeding women as all as the elderly, so that also these groups could be provided with access to new medicines once approved. Also, for indications with small patient populations, the European Medicines Agency together with the European HTA bodies, should continue and strengthen

[10] Miljković N, Godman B, Kovačević M, Polidori P, Tzimis L, Hoppe-Tichy T, Saar M, Antofie I, Horvath L, De Rijdt T, Vida RG, Kkolou E, Preece D, Tubić B, Peppard J, Martinez A, Yubero CG, Haddad R, Rajinac D, Zelić P, Jenzer H, Tartar F, Gitler G, Jeske M, Davidescu M, Beraud G, Kuruc-Poje D, Haag KS, Fischer H, Sviestina I, Ljubojević G, Markestad A, Vujić-Aleksić V, Nežić L, Crkvenčić A, Linnolahti J, Ašanin B, Duborija-Kovačević N, Bochenek T, Huys I and Miljković B (2020) Prospective Risk Assessment of Medicine Shortages in Europe and Israel: Findings and Implications. Front. Pharmacol. 11:357. doi: 10.3389/fphar.2020.00357.

[11] COM/2020/761 final. 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. Available from: https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:52020DC0761.

cooperation. In particular, the establishment of platform trials and the requirement that suitable drug candidates are tested in this environment rather than in isolated insufficient trials could be beneficial. Real-world evidence gathered through the monitoring of post-marketing safety and adverse events is being leveraged more and more. Real-world registries could consequently be useful since they can provide insights into the actual effectiveness of approved drugs as well as the long-term cost-effectiveness of these drugs, especially for special populations.

A way forward

Looking at both the barriers and the enablers, it is clear that there is no one-size-fits-all solution to meet the needs of all patients across Europe and that treatment enablers cannot be used in isolation. The different flagship initiatives and other actions of the 'Pharmaceutical Strategy for Europe' – through which some of the access to medicines issues experienced by patients should be addressed by 2025 – should take this into account. Instruments like Europe's Beating Cancer Plan and the HTA Regulation, whose adoption is pending, should be fully exploited in all treatment areas. Emphasis should be put on patient-centredness, a key element for the delivery of high-quality, effective and safe care. Cooperation and communication via a structural dialogue should be fostered not only in relation to medicines shortages but also with regards to other access to medicines issues since collaboration can contribute immensely to the improvement of health and care in Europe.

To help build the European Health Union that our patients deserve, **EAHP is committed to working together with the European institutions and other stakeholders by giving a voice to access issues that otherwise might be forgotten.** Unmet medical needs and access to medicines could for instance be better addressed through utilising the unique compounding skills of hospital pharmacists and their expertise in medicines procurement. Also, seamless transitions between the interfaces of different health settings need to be considered during the implementation of the Pharmaceutical Strategy to ensure that the patient care started in hospitals can be continued in the community.