THE LONDON SCHOOL OF ECONOMICS AND POLITICAL SCIENCE

Access to medicines in Europe

Delays and challenges for timely patient access

Executive summary

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Background

Various definitions of access to medicines exist; key aspects of access include, among others, affordability, availability and timely access.

The World Health Organization defines access to medicines using two main metrics: (i) availability, referring to the extent to which new medicines are available in the market for which they are intended, and (ii) affordability, referring to the extent to which prices of medicines are in line with the purchasing ability of healthcare systems and/or patients. Other definitions place more emphasis on the time to market access, rather than affordability, or on the patient access in the post-reimbursement phase of medicines, defined as "the phase that starts when the first patient is treated under a formal reimbursement scheme".

Access to innovative medicines varies greatly across European countries and remains a concern, especially for the therapeutic areas of oncology and HIV/AIDS.

Significant variations in access exist between European countries due to diverse regulatory settings, differing inclusion criteria for funding decisions, countries' purchasing power based on gross domestic product (GDP) per capita, healthcare expenditure, and pharmaceutical prices and utilization rates, among other factors.

Objective

The aims of this study are (i) to identify key factors that delay access across Europe, looking at the different stages of a medicine's lifecycle and focusing on oncology and HIV/AIDS, and (ii) to provide recommendations on key areas of focus that could ensure affordability and enable faster patient access and availability.

Geographic scope

The geographical scope of the research covers the 27 EU countries (EU27), Serbia and the UK.

Methods

An analytical framework identified the main policies/activities which can influence patient access across three main pillars of the access pathway:

a) horizon scanning, marketing authorization (MA), pricing policies, and Health Technology Assessment (HTA) informing pricing/reimbursement decisions.

b) decision-making on reimbursement and funding of treatments.

c) diffusion of treatments after they become available.

Macro-factors, such as economic and cultural factors, were also considered. Secondary evidence was identified through scoping reviews for each stage or theme, looking at what factors may

cause delays to patient access in Europe across the market access pathway and, where possible, for specific findings for oncology and HIV/AIDS.

Results

300 sources were downloaded and screened, of which 194 sources were included. Key access challenges identified across the different market access pathway steps include:

Marketing authorization

- Lengthy MA approval times because of clock-stops and/or long timeframes for opinions and decisions to be reached.

Pricing

- Command and control pricing systems, such as External Reference Pricing (ERP), or pricing systems with high administrative or evidence requirements.
- The potential for pricing systems (e.g. ERP) to encourage sequenced product launches.

Health Technology Assessment

- The timing of HTA processes, the length of the HTA process itself, and the time it takes for a positive HTA recommendation to be translated into a funding decision and for a funding decision to be implemented.
- HTA conducted at multiple levels (e.g., national, regional and/or hospital level) may result in differential or fragmented coverage and access.
- Involvement of multiple national HTA and reimbursement authorities in negotiations increases the workload with potential impact on timelines and access.
- Cross-country variation in HTA systems and negotiation processes results in diverging access outcomes for the same medicine in different countries.

Reimbursement & funding mechanisms

- While Managed Entry Agreements (MEAs) are generally considered positive for patient access, their expiry might prove challenging in terms of continuing patient access.
- Rigidities in reimbursement mechanisms and a lack of capacity and/or established legal or regulatory frameworks may impede adaptation to new data and uptake of innovative funding models that would otherwise have the potential to facilitate access to treatments.

Market uptake & diffusion

- Geographical factors (location of patients and care provision), as rural locations may experience slower adoption of new drugs when compared to urban environments.
- Health system and macro-level factors (e.g., the way the system operates, income level and market size). For specific therapeutic areas willingness to pay, and societal priorities may also contribute to patient access.

- Physician adherence to clinical guidance and, specifically, the introduction of new therapies.
- Social stigma around specific disease or against certain groups in society may impact how those diagnosed with certain conditions access treatments.

Considerations for policy change

This study highlights continued access issues across all stages of the market access pathway, and across countries despite the introduction of many schemes and initiatives introduced to optimize the pathway. The evidence points towards the key role of country, region, or policy specific contexts in determining whether a potential negative impact on access results from key stages in the access pathway.

Considerations for policy change and the improvement of major issues in patient access were drawn for each stage of the market access pathway, aiming to provide guidance on short-, medium- and long-term options to overcome challenges to access in Europe and in the context of the upcoming Pharmaceutical Strategy. These recommendations will not apply fully across all countries in Europe. Equally, any intervention may not operate individually or as a standalone solution as the various aspects of the access pathway are closely interlinked and often influenced by each other. Therefore, the following recommendations are presented here as a broader set of salient observations and respective suggestions towards improved access that arise from our analysis.

Regulation

- Focus on horizon scanning and early dialogue to maximize the ability and impact of authorization pathways designed for early patient access.
- Strengthen cooperation between regulatory agencies and national HTA agencies through the establishment of parallel review processes.

Pricing

- Improve the design and implementation of pricing systems and move towards pricing policies that are less administratively complex and consider the therapeutic value new medicines.
- Where necessary, modify certain elements of ERP systems to avoid spillover effects.
- Pricing policies promoting evidence-based price-setting should be implemented in the context of highly specialized therapies.
- Review whether pricing and reimbursement systems allow the greatest value to be derived from novel treatments.

Health technology assessment

- Monitor the time taken for the completion of HTAs and funding decisions.
- Improve coordination of HTA processes in countries with decentralized systems.

- Create special assessment pathways or tailor-made criteria for the evaluation of highly specialized treatments usually associated with high degree of uncertainty due to limited evidence.
- Invest in the infrastructure and development of joint mechanisms for RWE generation and EU-wide registries to address evidentiary gaps and/or in instances where significant uncertainty occurs during the assessment and appraisal process.
- Leverage current cooperation across European countries for health technology assessments.

Reimbursement & funding mechanisms

- Use MEAs or novel funding mechanisms to secure funding in cases of negative reimbursement decisions, particularly in areas of significant unmet need.
- Rely on RWE during reimbursement negotiations, especially considering incomplete evidence across EU countries, to ensure evidence required under MEAs is provided in a timely manner.
- Establish therapeutic area-specific solutions for data generation, budgeting, or other bottlenecks.

Market uptake & diffusion

- Ensure clinical guidelines and or care pathways are as up to date as possible.
- Collect evidence on country-specific factors impeding diffusion of medicines.

Macro-level factors & wider system needs

- Minimize intra-country variations, which may result in inequitable access and population-level disparities.
- Ensure accurate and timely diagnosis and treatment initiation through accessible screening and diagnosis programs and services for key disease areas.
- For HIV in particular, it is important to have measures in place at national/community level to reduce criminalization of HIV and/or related activities and social stigma.
- Conduct and sponsor more research into determinants and hurdles for access to support the refinement of existing tools and the use of novel solutions where possible.
- Strike the right balance between health policy and industrial policy.
- Political will is essential to ensure regulatory pathways and value assessment encourage new medicines to come to market.
- Healthcare systems are constantly and dynamically evolving and need to be able to continuously adapt to changing circumstances.

Conclusion

Patient access remains a challenge for many countries, centered around availability, affordability, time impact, and geographical variation challenges. Time delays occur given the complexity of the access pathway and the multiplicity of stakeholders involved. Unequal access

across countries can also result due to the effects of different policies, different health system designs, socioeconomic and cultural factors. Strategic efforts to overcome tensions in the market access pathway include the availability of schemes such early dialogue, early scientific advice and parallel review, harmonization of evidentiary requirements and common ways to deal with uncertainty and the use of novel funding mechanisms where possible, to ameliorate the availability and affordability of medicines. An improved evidence base for specific aspects of the pathway and for disease areas (such as HIV/AIDS) is needed to allow for monitoring of current systems, informed policy-making, and support both amendments to existing tools and the use of novel and groundbreaking solutions.

In order to reduce the challenges and tensions in the pathway and improve the affordability and availability of medicines across and within countries, decision-makers, governments and purchasers of medicines should ensure that regulatory, pricing and reimbursement processes can adapt to the fast-paced and highly innovative health environment and ensure better health outcomes for patients across countries.