European Alliance for Vision Research and Ophthalmology

EIP ON AHA
PARTNER

Pharmaceutical Strategy
Timely patient access to affordable medicines

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Executive Summary.

The EU EYE welcomes the initiative Pharmaceutical Strategy with its multiple goals on equity in access to safe medicines; incentivise innovation; environmental impact of medicines and strategic autonomy. The Strategy can achieve a shift towards health outcomes-driven healthcare systems and support national health systems of small and lower income countries to address existing information asymmetry and inequalities in negotiating power. The Strategy needs to be complemented by:

- a broader dialogue across the entire healthcare ecosystem to align innovation efforts to public health and health systems' needs;
- an incentive framework to stimulate engagement of all stakeholders of the healthcare ecosystem on equal footing and to address clinical needs, access, innovation and environment with financial and non-financial commitments of all Member States and
- coordination along the policy continuum to address current conflicting interests among interlinked policy frameworks.

The EU EYE believes that strengthening the interaction with patients and healthcare professionals as the end users in the pharmaceutical supply chain should focus on co-creation in:

- the co-creation together with relevant medical organisations of a list of medicines which can be considered 'essential when in shortage'; including a grading/ranking system in terms of impact on health when they are unavailable e.g. "life threatening" ; "potentially causing irreversible health damage" etc.
- communication structures linking such list directly to real world clinical settings for direct updates from across the EU countries.

The Pharmaceutical Strategy can be pivotal in spreading worldwide the EU value and perspectives to the right to health, provided the strategy is complemented by:

- a continuum of actions aiming to strengthen regulatory sciences, to support regulatory scientific advice and to constantly evaluate and monitor policy implementation and its impact (existing incentive schemes, orphan medicines, generics/biosimilars, off-label use, etc);
- a wider reform that creates consistency and complementarity by addressing current conflicting interests among interlinked policy areas - trade policy, IP protection, free trade and public health;
- broader transparent dialogue that focus R&D investment on societal benefits by delinking it as much as possible from financial motives.

The recent pandemic demonstrated a need for rapid knowledge sharing when redirecting medicines to a new use. Despite EMA's warnings on lack of evidence regarding benefits, attempts to redirect hydroxychloroquine and chloroquine as antiviral agents against COVID-19 proceeded in high numbers and uncoordinated. An on-demand repurposing framework, as a hybrid between the piloted repurposing framework and the European Reference Network model, can optimise rapid sharing on outcomes and clinical experience. Such framework can be hosted within the ordinary repurposing framework and defined by: the capability to be activated at times of emerging threats such as new infectious diseases; and a coordinating body to overlook such efforts, preferably drawn from within the current regulatory community such as EMA's pandemic Task.

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1. A Broader Dialogue across the entire healthcare ecosystem

European associations and learned societies are currently underused as a resource in the decision-making process of the European Union, particularly in removing any sponsorship bias in the evidence base that may overplay benefits and underplay harms.
More importantly the regulatory system can tap into the potential of disease registries and large-scale trials run by numerous European consortia and medical associations. A number of them have been established by the ophthalmology community: European Cornea and Cell Transplantation Registry https://www.ecctr.org, European Eye Epidemiology, E3 http://www.eye-epi.eu; EUREQUO https://www.eurequo.org; EGS Registry, https://www.eugs.org/registry/.
Strengthening the communication channels to such entities will ensure a fast, regular and coordinated transfer of information on outcomes directly to regulatory bodies. This is particularly important for the training of AI systems which are constrained to learning from available observational health data. The merging data from large epidemiological trials can provide valuable training population-representative datasets to support robust conclusions with the potential to improve eye health outcomes at population level.

2. Coordination along the policy continuum and collaboration at global level

The Pharmaceutical Strategy will be more effective in achieving timely access to medicines if there is:

- consistency and complementarity with other EU policy frameworks and legislative actions such as the medicines repurposing framework and the HTA legislative proposal;

- concurrent revision/updating of other related frameworks such as the Directive 89/105/EEC ('the Transparency Directive') with focus on price-setting procedures and reimbursement systems taking into account the challenges of the market;

- synergy with global regulatory efforts on the timely entry of medicines in the market such the ongoing work of the International Council for Harmonization (ICH) on harmonizing standards in generic drug bioequivalence.

Repurposing. On a broad level, the EU EYE is of the opinion that the Pharmaceutical Strategy could take the opportunity to address barriers encountered in drug repurposing in national efforts until now[1] and create space for objective data generation for new labelling (repurposing) through:

- balanced and proportional incentives for both data generation by researchers (independent/academic) and for marketing authorization holder(s) to apply for a variation, extension, or new marketing authorization;
- support the data generators with dissemination of scientific advice briefings and training programmes on regulatory procedures.

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[1] Giovannoni et al., 2015; POLICY AND PRACTICE REVIEWS ARTICLE; C Verbaanderd et al. Front. Pharmacol., 31 January 2020 |https://www.frontiersin.org/articles/10.3389/fphar.2019.01664/full
On-Label or Off-Label? Overcoming Regulatory and Financial Barriers to Bring Repurposed Medicines to Cancer Patients; https://www.frontiersin.org/articles/10.3389/fphar.2019.01664/full

Repurposing - On Demand model. The EU EYE believes that one specific action within the repurposing efforts will be of great benefit to the aims of the Pharmaceutical Strategy.
The recent pandemic has demonstrated the need for rapid knowledge sharing on both positive and negative outcomes when redirecting medicines to a new use. Despite the warnings by the EMA on the lack of evidence regarding benefits, the attempt to redirect hydroxychloroquine and chloroquine as antiviral agents against COVID-19 proceeded in high numbers and uncoordinated. Supported largely by emerging belief that "crisis situations demand exceptions to high standards for quality" in research, testing of different regimens derived from a common clinical hypothesis proceeded in uncoordinated protocols with huge amount of duplication as individual countries conducted multiple clinical trials instead of the customary one or two high-quality ones. Such attempts led to confusion, over-treatment and have put the safety and well-being of patients at risk as the probability of false-positive findings due to chance was increased and cross-comparisons were made impossible.

A repurposing framework is currently piloted across the EU with the aim to support smaller actors such as academic/research institutes or non-profit/foundation entities (the so called *champions*) in exploring new uses for existing medicines[2]. The EU EYE proposes that such efforts include a discussion in the benefits of an *on-demand repurposing* framework that is hybrid between the piloted repurposing framework and the model of the European Reference Networks. The *on-demand repurposing* framework could optimise rapid knowledge sharing on treatment outcomes and clinical experience across Member States and enable the rapid identification of promising treatment approaches in urgent situations such as during outbreaks of emerging infectious threats. It can be hosted within the ordinary repurposing framework and be defined by:

- the capability to be activated at times of emerging threats such as new infectious diseases
- and by having its own coordinating body to overlook such efforts, preferably drawn from within the current regulatory community such as EMA's pandemic Task Force.

3. Transparency and shared social responsibility to improve access to medicines

There is a need for a sustainable, transparent and stable system that provides clear key guidelines to maximise the benefits of the pricing policy. Systematic monitoring of policy implementation and effectiveness of policy measures (patent reform legislation, orphan drug programs, pricing regulations/strategies, incentivizing the use of generics) must be carried out by bodies independent of commercial interests with accountability and transparency regarding data collection process and analysis to inform pricing decisions and incentives for innovation particularly in medicines for children.
Studies have shown that the heterogeneity in the external reference pricing (ERP) design and the calculation methods used to compute medicine prices have policy implications across the Member States of the EU[3] such as launch delays in low-income countries, parallel trade reducing drug stock levels, inflated prices in low income countries, reduced incentive for continued R&D and reduced access to medicines in some regions. The lack of reliable sources of price information, price heterogeneity, exchange rate volatility, and hidden discounts limit ERP implementation.

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[2] https://ec.europa.eu/health/sites/health/files/files/committee/stamp/stamp_9_40_1_en.pdf

[3] http://www.lse.ac.uk/business-and-consultancy/consulting/assets/documents/the-implementation-of-external-reference-pricing-within-and-across-country-borders.pdf

Decisions on pricing and reimbursement should be informed by adequate and robust data on the real therapeutic added value of new medicines as assessed on the basis of clinical, economic and social benefit criteria (as compared to best available alternative). Measuring the quality and quantity of life generated by a drug (value-based pricing) has hidden risks in the absence of a method of determining what would count as evidence of value: it is open to manipulation in addition to the moral dilemma regarding the actual worth of medicines (open-ended or capped).

The European Alliance for Vision Research and Ophthalmology (EU EYE) is a non-profit pan European advocacy group. We draw from the experience of our ophthalmology community across Europe to provide a forum for the integration of research priorities, policies and strategies in eye healthcare and vision research.

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