6th EPHA Universal

ACCESS TO MEDICINES FORUM

EVENT REPORT

8 December 2023

For more information and a detailed schedule please visit the event page:
www.epha.org/events/a2m-forum-2023/

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INTRODUCTION

The Access to Medicines (A2M) Forum, hosted annually by EPHA, served as a platform for facilitating discussions regarding pharmaceutical and medicine policies in Brussels and beyond. The A2M Forum contributed to the capacity-building for different stakeholders (patients, healthcare professionals, payers, medicine agencies, European policymakers, health law experts) on the topic of access to medicines.

The 2023 edition of EPHA's A2M Forum provided an opportunity for EPHA's members and partners of EPHA's hosted **European Alliance for Responsible R&D and Affordable Medicines** to exchange with other stakeholders. The revision of the EU pharmaceutical package brings unique opportunities to restore the balance between the public and the private interest and ensure affordable access to quality, safe, innovative, and effective medicines.

EPHA, its members and partner organisations have been at a centre stage in contributing to policy debates, forging new coalitions, highlighting the public health perspective, and ensuring more inclusive and balanced discussion around EU pharmaceutical policies.

WELCOME REMARKS

Dr Milka Sokolović, EPHA's Director General provided welcome remarks, highlighting EPHA's commitment to the engagement on the EU Pharmaceutical Strategy, which touches deeply upon access to medicines and effective incentives. She introduced the event with the keynote speaker from World Health Organization (WHO) European Region to set the scene, followed by two sessions with expert panel discussions. She stressed the importance on the upcoming debate on transparency and the limitations of the proposed EU Pharmaceutical Strategy, reminding us of the underlying value of the upcoming debate to consider equity as the basis for the discussions on affordability and access.

SETTING THE SCENE

Sarah Garner, WHO Senior Policy Advisor on Access to Medicines and Health Products from the WHO Regional Office for Europe set the scene on the future of antibiotics and pharmaceutical innovation and provided reflections on the EU pharmaceutical package.

Gaps in the EU landscape on pharmaceuticals were touched upon and concrete ongoing initiatives from WHO Europe were shared. She reminded the audience that the health care systems in countries are under crisis. To solve this crisis, WHO Europe has created the WHO Novel Medicine Platform (NMP).

A short background on the WHO Regional Office for Europe was presented. Established in 1978, covering 53 Member States, accounting to 900 million inhabitants. It represents an enormous market and can serve as a peer versus the US market.

The creation of the NMP was detailed. It started as the Oslo Medicine Initiative in 2020-2022 which identified the need to bring partners together. Currently the NMP has diverse members with 50 Member States, 25 Nongovernmental Organizations (NGOs), 6 industry business associations and 18 partners (EU, OECD,..). The NMP is operational through four Working Groups (WGs): WG 1 (transparency), WG 2 (solidarity), WG 3 (sustainability) and WG 4 (antimicrobials). EPHA is a member of the NMP.

Lastly, the WHO EURO Antimicrobial Resistance (AMR) Roadmap, adopted in Astana, Kazakhstan in October 2023 was mentioned. The AMR Roadmap represent the WHO's European Region vision and looks at priorities how the Regional Office will approach AMR and access to antimicrobials. She thanked EPHA for its strong commitment and engagement in the design and launch of the roadmap and continued to ask for engagement as a community to move forward together.

> "WHO Novel Medicine Platform (NMP) can bring change. Together, we can make that change."
>
> **Sarah Garner,** WHO Regional Office for Europe

DEVELOPMENT AND ACCESS TO NEW ANTIBIOTICS: INNOVATIVE AND FAIR INCENTIVES FOR A RESILIENT FUTURE

The first panel session focussed on the need for new antibiotics to combat antimicrobial resistance (AMR) and the experts' discussed concerns regarding the European Commission's proposed transferable exclusivity voucher (TEV) as a costly and indirect pull incentive.

The session explored alternatives, more effective incentives – such as market entry rewards, revenue guarantees, and R&D milestone payments – to promote antibiotic development and ensure access to existing ones. Equally important is to ensure timely access to existing antibiotics along with stewardship measures to ensure antibiotics are used prudently. In the proposed revision of the EU pharmaceutical legislation, the EC has put forward a proposal to create a new pull incentive to spur the development of new antibiotics. However, as argued by EPHA and by 14 member states, the transferable exclusivity extension (voucher) would create a very costly, non-transparent and indirect pull incentive to spur the development for new antibiotics.

The first expert, **Dimitra Panteli** Programme Manager from the European Observatory on Health Systems and Policies (hereafter Observatory) gave an overview of the key findings of a **policy brief** published by the Observatory at the request of the Swedish Presidency of the Council of the European Union. The policy brief "How can the EU support sustainable innovation and access to effective antibiotics?" looked at policy options for existing and new medicines. The major challenges across the research and development pathways for antibiotics were unveiled. A WHO review of antibiotic pipeline (newly approved and under development) pointed out that only a handful of new antibiotics have been developed and launched over the last few decades, and almost none have any innovative characteristics. She concluded that the clinical pipeline is insufficient.

She touched upon key scientific, economic, structural, and regulatory barriers with regards to development of novel antibiotics with innovative characteristics. The expected return of investment is low. Most large pharmaceutical companies have withdrawn from antibiotic research and development (R&D) for two main reasons: (1) high risk of failure and (2) antibiotics are less profitable. Academia and Small and Medium-sized Enterprises (SMEs) now drive antibiotic R&D however they face difficulty securing funding for pre-clinical and early clinical trials, and risk significant economic losses when launching new antibiotics. Challenges were mentioned such as lack of secured access and inconsistent access across the Member States.

Overall, she summarized two main drivers behind access to new innovative antibiotics: (1) financial considerations and (2) the fragility of antibiotics supply chain (single source APIs, few generic manufacturing), which often lead up to shortages.

Solutions were offered through a holistic package of incentives which include three main pillars: (1) push incentives, (2) pull incentives and (3) aligning incentives. Push incentives (direct funding and grants) can reduce the cost of R&D. Pull incentives (direct financial pull such as the Netflix model) or indirect model (vouchers) could increase potential revenue. Most importantly is to align the incentives.

To conclude, the One Health Approach is the urgent action needed. The European Union (EU) has a major role to play in combatting antimicrobial resistance (AMR) and in contributing to global solutions.

> "The EU is the second largest pharmaceutical market and needs to act alongside others in the G7/G20. We may focus today on prices and access, but we need to keep the broader view in mind".
>
> **Dimitra Panteli,** WHO European Observatory on Health Systems and Policies

The second panel expert, **Dimitri Eynikel** representing Doctors Without Borders' Access Campaign shared the challenges on global access to medicines, vaccines, and diagnostics. Field impressions on the rise of AMR were given. Examples of situations in Iraq, Syria, Yemen, Niger and DRC where war-wounded or sepsis cases cannot be treated anymore illustrated the rise of AMR. The global spread and the conditions that fuel the rise of AMR are highly prevalent in low and middle income (LMI) countries in particular limited application of infection, prevention, and control measures.

Secondly, shortages of antibiotics and lack of access to diagnostics and vaccines are all elements present in LMI countries. There is need for a global response to the crisis that surpasses all elements. Foster an innovation model and scale up to work globally.

> "We need to consider major challenges with access to existing antibiotics! Every year, 5.7 million people die because of access".
> "Addressing AMR will need to address inequity"
>
> **Dimitri Eynikel**. Doctors Without Borders' Access Campaign

The third panel expert, **Helle Aagaard**, Director from ReAct - Action on Antibiotic Resistance illustrated how antibiotic resistance is an overwhelming challenge. The risk is to lose the overview and the bigger picture. Different elements should be addressed simultaneously. The EU pharmaceutical legislation should serve to sustain access. A problem analysis should form the starting point of debate. Large pharmaceutical companies are pulling out and now small SME are in the lead. This equation should not form the starting point. It is a political debate, but the TEV will not solve the issue, the questions to ask are on ensuring sustainability and affordability of antibiotics.

> "Big pharmaceuticals have left, no, that is the wrong question. You need to address different questions. Have the end goal set!"
>
> **Helle Aagaard,** ReAct

Rosa Castro, Senior Manager Development and Health for Deutsche Stiftung Weltbevölkerung (DSW). reflected on her experience with EPHA, the Alliance and now DSW. The global dimension of the EU pharmaceutical legislation should not be forgotten. The EU's model will count outside in the world. It is important for the EU to recognize that impact as the biggest burden remains in LMI countries.

The EU pharmaceutical legislation and AMR are global health threats. AMR has been a neglected disease. Within the EU's proposal sit the vouchers, which are not assessed within the context it will be applied. Vouchers have been utilized in other parts of the world with a high cost, this should be assessed as part of the toolbox. The push and pull incentive should be part of the broader picture.

Good alternatives such as the pilot study developed by DG HERA and the European Council Recommendations on AMR were reflected upon. The amendments of the EU pharmaceutical legislation will hopefully contain these alternatives.

> "Innovation and access are a difficult mix to combine"
> "Poverty related and neglected diseases, there is no commercial incentive to invest."
>
> **Rosa Castro**, DSW

Tamsin Rose, the moderator opened the debate and asked the experts to share their perspectives on the most preferred incentive structure to be put forward by the EU.

The experts agreed that alternatives under revision such as the HERA study look promising. Helle from ReAct referred to the pull incentive (milestone prizes), a study by the Swedish looking at milestones' prizes and the vouchers come out worst. Dimitri asked to rethink about the question, it is not the incentives that will solve the problem. We should be asking what is needed to deliver and for whom? Multiple incentives are needed. The burden is high on LMI countries. Late-stage clinical trials are taking place in LMI countries. High prevalence cases (sepsis patients, children, pregnant women) should be part of the clinical trials and the TEV will not solve that problem.

A reference was made to the Global Antibiotic Research & Development Partnership (GARDP), which accelerates the development and access of treatments for drug-resistant bacterial infections. GARPD is currently doing R&D and licensing to local producers. The cost of 440 million euros for one voucher and GARDP is a low for-profit model proposing 220 million euros for the next five years to develop new antibiotics, therefore the GARDP model should be explored. Dimitra from the Observatory confirmed the incentive on delinking revenue from sales. The structure of the incentives should be well understood, reference was made to the Advance purchase agreements with the vaccines during COVID, which was one joined pull incentive.

The strong voice of the European Union can count at global level through the EU' role in G7 and G20. A good starting point of the global mandate, anchored in the EU global health strategy, where AMR is part of the priorities. Rosa from DSW, agreed with the EU taking a leadership position on AMR and having a dialogue with the rest of the world. The need for a mix of push and pull incentives was agreed upon. To put all the resources in the TEV is not recommendable. More data should be shared on the cost for developing antibiotics.

An exchange with the audience took place on incentives, detailed discussion on TEV as an exclusive model. Reference to the Study prepared by the European Parliament Science and Technology Options Assessment (STOA) which stated that TEV works for innovation purposes, but it does not work for unmet needs. Majority of the audience agreed to continue to pressure the EU to remove the TEV and ask for an impact assessment of the TEV, looking at more alternatives (study from DG HERA, player pay model).

To conclude, our panellist warned against the false narrative and reminded that the EU needs a system that will function and work. The EU Pharmaceutical legislation is only one piece. The debate is global. Keep the global perspective and ensure that EU interventions contribute to the global solution. The United Nations General Assembly High level meeting on AMR is scheduled to take place in September 2024, EU voices should not be limited.

PAVING THE WAY TOWARDS FAIR MEDICINE PRICING: ENSURING TRANSPARENCY OF R&D COSTS

The second panel session debated on the transparency regarding the real R&D cost for a new medicine. And when a medicine has been developed with public funding's support, should companies be able to freely choose the price that extracts the higher profit for them? Fair prices for medicines have been defined as "justifiable, predictable and cost-effective within the aims and priorities of the healthcare systems and the available budget". This requires consideration of the needs of patients and health systems, the affordability of treatments and the balance between the cost of bringing a product to market and its final price. Hence, transparency of pharmaceutical R&D costs is a necessary step towards fair(er) prices. Without a clear understanding of the costs to develop a new medicine, the quest for fair prices remains elusive. The revision of the EU pharmaceutical legislation has opened the opportunity to request information from pharmaceutical companies about the public funding contributions they have received for the development of a new medicine. However, the proposed text is limiting the disclosure of information to direct public contributions. A too narrow scope for this obligation risks being incomplete at best or counterproductive at worst. This session convened experts from different areas discussing ways forward to improve transparency of pharmaceutical R&D costs in the context of the revision of the EU pharmaceutical legislation.

The first speaker of this panel session, **Anne Hendrickx**, Advisor in the Research department of Solidaris (Belgian mutual fund, speaking as a member of The International Association of Mutual Benefit Societies (AIM)) shared details on the 'Fair pricing calculator'. She reminded us on the question, **can we have fair prices if we do not know the R&D costs?** Anne presented work ongoing on definition of a fair price as part of a Horizon Europe project: "ASCERTAIN: Affordability and Sustainability improvements through new pricing, Cost- Effectiveness and ReimbursemenT models to Appraise iNnovative health technologies consortium." As most definitions (except the industry's), WHO's concept of fair pricing for medicines includes R&D costs. AIM's 'fair pricing calculator' is a tool designed to help healthcare stakeholders or anybody interested in the matter to calculate a FAIR price for new or existing medicines (without generic competition) and compare it to the price paid or being negotiated. It is a hands-on tool with clear proposals for data components to contribute to European and international debates about fair pricing and the transparency of R&D costs of medicines. The model is based on a simple and transparent algorithm: the European fair price would cover the costs of R&D and production, allow a justified but limited amount of expenditure on sales and medical information, offer reasonable profit, and grant an innovation bonus for medicines with an added therapeutic value. However, the R&D component is the most complicated. Unless transparency on R&D costs is provided, it is proposed to use a lump sum for the R&D component (of 250 million). If real costs are used a cap (of 2,5 billion) to the real development costs of a new drug is used in order to avoid inflated prices. There is still a difficult trade off on what is considered as R&D cost: Anne provided an example of Gilead which acquired Pharmasset for 11 billion in 2011 but Pharmasset only spent 271 million in R&D in the whole period from 2003-2011, so what is the amount to be considered as R&D cost? R&D cost is a difficult parameter that would benefit a lot from transparency. Take the example of a Hepatitis C drug (AIM price), the fair price calculation gives a price of the drug of 905 euro/patient if real costs (estimated at 800 million) are used. But in the absence of transparency, the lump sum of 250 million euro would be applied and the fair price per patient for that drug would only be 435 euro. If we want to discuss R&D, transparency is needed.

> "Transparency on total R&D is a prerequisite for fair prices."
>
> **Anne Hendrickx**, Solidaris

The second expert in this panel was **Suzannah Chapman**, Health Policy Analyst in the Health Division Directorate for Employment, Labour and Social Affairs from the Organisation for Economic Co-operation and Development (OECD). The OECD undertook a feasibility study in August 2023 to establish a set of indicators to measure performance and resource allocation in the pharmaceutical industry. The results confirm the feasibility of creating a set of indicators for the routine monitoring of pharmaceutical industry inputs and activity. The study looked at revenues, activities, and use of resources (financial and non-financial) and outputs (clinical benefits). The study also highlighted the need for careful analysis and thoughtful interpretation to fully appreciate the implications for policy makers. If you look at aggregated results, aggregates are not representable as they are driven by bigger firms. In the report, across the data in 26 OECD countries the pharmaceutical industry receives 10 % of the inputs from external sources (which includes public funding). Of that, 10 % goes directly in R&D (input) and produces 40% R&D (activities) but these are estimates and we need to be careful as the 10% is driven by the US data. We should disaggregate more and do the analysis underneath.

> "In terms of policy implications, we are not there yet. We are trying to harmonize the facts. We should look more at outputs points, are we developing the drugs that are efficient for patients."
>
> **Suzannah Chapman**, OECD

The third speaker, **Javier de la Cueva**, a lawyer specialised in free intellectual property licenses from Complutense University of Madrid was clear about the need to produce more research. His request is tailored by the absence of relevant data and the data available is biased. Absence of data is linked to the misinterpretation of Intellectual Property (IP) rights. The term IP comprises four major fields: copyright, patents, trademarks, and trade secrets. Trade secrets consist of information that is valuable because it is not generally known. We have pushed for transparency. The research conducted lines of action to compare the prices of drugs and analyse the Spanish administration decision taking on price setting. It is challenging as it seems the government is taking a favourite side for the pharmaceutical industry. As a result of our research, we took the Spanish administration to court and did a freedom of information petition. We found in the two cases the same strategy of the different companies - They do not want share information. They cut the knowledge. No parameters. As a results, these led to two resolutions in Spain enforcing pharmaceutical companies to disclose the data, but the case is now in appeal court.

> "We are dealing with data that is coming from a criminal concept."
> "It is their business model, the absence of data."
>
> **Javier de la Cueva**, Lawyer

The last speaker of the panel session was **Anna Prokůpková**, Health Policy Advisor from the Greens/EFA Group at European Parliament. She provided an update on the timeline and progress of the EU Pharmaceutical legislation. She reminded the audience that price setting is a Member State competence. However, the EU can do action in this field and developing a cost-price model would be an example of action to take. To come up with a cost-price model, transparency of costs for development of pharmaceuticals is needed. The EU's draft pharmaceutical legislation contains elements around transparency with regards to the funding of R&D of medicinal products. Article 57 of the proposed medicines Directive will require marketing authorisation (MA) applicants and marketing authorisation holders (MAHs) to publicly declare any "direct financial support received from any public authority or publicly funded body" in relation to "any activities for the research and development of the medical product" covered by a national or centralised MA, irrespective of which legal entity has received the support. The obligation is not restricted to only EU financial support, any funding received from public authorities and publicly funded bodies located outside of the EU should be transparent. At EU level, the responsibility sits within evidence generation. The European Parliament is in the midst of negotiation and make standards. It is crucial to address the evidence gap as there is new drugs to be marketed where the added value is not known. Fair price is linked to the market, so it is important to contextualize if the market for the specific drug is a monopoly or regular market.

> "Open science is key but when it comes to details, it is more difficult when it is company data, and everyone is screaming for more protection of data."
>
> **Anna Prokůpková**, Greens/EFA Group

After the panel discussion, the moderator opened the floor for question. A few audience members picked up on the "use of the law", to request more transparency.

Anna from the Greens/EFA Group confirmed that the Law has been used as they pursued the Commission in the European Court of Justice and if the case is won it will the first case law. There is the financial regulation that companies can protect their information but there is an overriding public interest for disclosure. It was suggested to indeed explore the possibility of using the law more.

Javier also confirmed that IP rights are not solid rock and should be granulated. Reference was made to a report commissioned by Directorate General for Research and Innovation in 2022 on Open Science and IP rights. The EU approach to IPR legislation has four main components: (1) Copyrights, (2) Patents, (3) Trademarks and (4) trade secrets. But when we speak about health trade secrets should not be allowed. IP is not applicable and therefore transparency and the argumentation of IP. Trade competition should be part of trade secret. We need to bring things to reality. Sociology of science, what are they doing with a conference in Las Vegas, is this RD? More of the budget goes in marketing. If we are going to analyse all what is in a price, the activities should not include champagne and prostitution. We must speak of sociology of science.

CLOSING REMARKS

Firstly, the WHO Novel Medicine Platform was explained, the opportunities it brings to work together on actions.

Secondly, the evidence towards the needs for new antibiotics were presented. Time is of essence along with stewardship measures. The equity aspect should form the basis of our actions. The starting point of reflection should be: “How to achieve equitable access to all”?

It was made clear that the debate on access to medicines is of global dimension and the global health threats cannot be sorted out by an EU centric approach.

Thirdly, Costs using TEV were discussed and the lack of evidence to use the TEV in Europe. Milestones and alternative solutions were presented such as the DG HERA study.

The conclusion of the first session - where there is a need for political will and funding to move forward, the moment is right. There is enough political momentum for us to drop TEV.

In the second session, prices, and lack of transparency of R&D cost of new medicines were the focus of debate. How is it possible in Europe, that public fundings of R&D is facilitated but still private sector has full flexibility in price setting. Fair prices of medicines were presented as a solution but the balance between the costs and its final prices does not sit right. The revision of the EU pharmaceutical legislation presents the opportunity to advocate for the inclusion of transparency and fair price setting.

Open science solutions, cost-based and value prices model were shared. The EU values should be enshrined in the EU pharmaceutical package.

> "Are we late for the sailing boat. We are not, we can still address unmet needs. We need to base it on our values, for all. To close, as EPHA and with the Alliance, we are ready to engage and work together in 2024."
>
> **Milka Sokolović,** European Public Health Alliance

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