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Innovations in pharmaceutical policies and learnings for sustainable access to affordable medicines

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ABSTRACT

Sustainable access to affordable medicines remains a public health issue globally, including for high-income countries. To foster the debate on avenues for the future, the fifth PPRI Conference held in Vienna on 25 and 26 April 2024 will offer a forum for the debate on innovating pharmaceutical policymaking to develop and implement futureproof policy options, which are able to address current and future challenges. The Conference invites a broad audience of stakeholders, including researchers, policymakers, payers, patients, industry and health professionals. The conference topics are organised in three strands: Strand 1 on 'Local challenges, global learnings' aims to contribute to lively discussions on the implementation of pharmaceutical policies across the globe. Best-practice examples will be presented, supplemented by case studies of less effective policies which can offer rich learnings. Strand 2 on 'Strengthening the evidence base' is the place for presentations and discussions on topics such as health technology assessments, managed entry agreements and real-world data. Strand 3 'Futureproofing pharmaceutical policies' is particularly dedicated to explore innovation in policymaking to achieve sustainable access to affordable medicines.

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All over the world, policymakers are struggling to ensure sustainable access to affordable medicines. Though progress in life sciences has saved and improved many lives, millions of people cannot benefit since they lack access to medicines for the treatment of acute or chronic diseases. Innovative medicines have been developed, authorised and launched in some markets, but these medicines are not accessible to all patients who need them. Not all newly authorised medicines bring additional health benefits. Many disease priorities still lack therapeutic innovations.

The sustainability of publicly funded health systems is challenged by increasing pharmaceutical expenditure, which is frequently driven by spending on a few medicines with high prices or high volumes. Advanced therapy medicinal products (ATMPs), such as gene and cell therapies, are offered at premium prices that even governments in high-income countries cannot afford. Health systems in low- and middle-income countries are often not equipped to deliver such treatments. A trend towards labelling therapies as orphan medicines to target high-priced therapies to very small population groups has been observed. In oncology, for instance, it was shown that despite providing little added benefit pharmaceutical companies manage to recoup research and development costs within few years (Brinkhuis et al., 2024). Markets which are considered sufficiently attractive for suppliers are being served while less attractive markets are either not served, or may only be served with significant delays and less priority. Lack of price regulation, including in the supply chains, and limited coverage in public funding systems can result in high out-of-pocket payments for patients and their carers (Wirtz et al., 2017). This, in turn, may force them to have to choose between the needed medication and other essential household goods, to purchase (lower-priced) poor-quality medication or to even refrain from medicine use. Well-established medicines which have been marketed for decades suddenly 'disappear', with no or only high-priced alternatives being offered instead. Medicine shortages continue to increase and have become a major issue, also in high-income countries.

The COVID-19 pandemic has highlighted the inequity in global access to life saving medicines and technologies and the ubiquitous vulnerability of supply chains. However, the pandemic also accentuated the importance of public health, including the need to establish resilient and robust health systems and to have a comprehensive policy toolbox, which can be further adapted to address upcoming needs. It has been acknowledged that policymaking should be flexible and innovative in responding to emergencies and new challenges and should encompass dialogue and collaboration with all stakeholders to achieve the right to health for all (Sagan et al., 2021).

The fifth Pharmaceutical Pricing and Reimbursement Information (PPRI) Conference held in Vienna, Austria, on 25 and 26 April 2024, takes place in a period marked by numerous significant challenges in access to affordable

medicines, as outlined above. These challenges can be an opportunity to identify learnings and areas for collaboration, in particular from the public health crisis during the pandemic.

Thus, the PPRI Conference 2024 is well-timed for taking stock of current developments in pharmaceutical policy around pricing and reimbursement, and for further exploring potential limitations and challenges as well as best practices and solutions for the future. This will be achieved through presentations of stringent scientific research, the dissemination of case study examples, the discussion of new and innovative approaches and policies, and the exchange of different country and stakeholder perspectives and lessons learned. The conference topics are organised in three different strands as outlined below.

Learning from each other

The current and previous PPRI Conferences are organised for a broad audience of stakeholders (researchers, policymakers, payers, patients, industry, health professionals) and complement the activities of the PPRI network of public authorities responsible for pharmaceutical pricing and reimbursement policies from 50 countries, mostly in the World Health Organization (WHO) European Region (Vogler & Zimmermann, 2022). Sharing of information within the PPRI network is focused on an exchange of hands-on experiences in practical policy implementation between peers that struggle with similar challenges (Vogler et al., 2014). The PPRI Conferences build on this approach, but offer additional perspectives from further countries not represented in the PPRI network and non-governmental stakeholders. They enable learning through lively discussions on policy implementation involving stakeholders from health systems with different levels of maturity and health coverage. While best-practice examples are always a source of encouragement for others, case studies of policies whose implementation has proven to be less effective or lack of policy implementation can offer particularly rich learnings (e.g., high medicine prices in the hospital sector in Austria, without price regulation, compared to the price-regulated outpatient sector (Vogler, 2022b)).

Offering policymakers exposure to public health researchers globally, the PPRI Conference will, in return, draw from scientific research and findings relating to pharmaceutical policies in numerous countries. Some of these policies may include a novel element in their methodological design, whereas other pricing, procurement and reimbursement practices may be perceived as being rather designed in 'textbook' style. Even in the latter case, their introduction is an important step towards improved sustainable access to medicines. An evaluation of policy implementation is highly welcome since it offers learnings for the respective country and others. Also, the importance of monitoring and evaluation of policies has been reiterated several times (WHO, 2020), but

these assessments are conducted far too seldom, and the publication of their findings in the international literature is even less frequent. The PPRI Conference aims to address this dissemination gap, and thus the Conference Supplement publishes findings of studies on national and local policy implementation in pharmaceutical pricing, procurement, and reimbursement, as presented in Strand 1 'Local challenges, global learnings'.

Strengthening the evidence base

The fourth PPRI Conference held in October 2019 concluded that 'evidence' and 'transparency' constitute two key components to ensure access to medicines through 'fair prices' (Vogler, Zimmermann, et al., 2021). While clinical progress and innovation is highly awaited, a reality check has shown limited clinical evidence and/or little added benefit for newly authorised oncology medicines (for which high prices are charged) (Davis et al., 2017; Vokinger et al., 2020). Discussion has emerged to explore the usefulness and potential need of real-world data despite their lower evidence grade. European Union (EU) Member States have been working on the implementation of Regulation (EU) 2021/2282 on health technology assessment (HTAR), which provides for joint clinical assessments of defined medicines in the EU from 2025 onwards. Adding to the challenge of organising collaboration in HTA across countries, questions also arise around a common understanding of evidence.

Access to (different types of) information helps policymakers to take better informed and more evidence-based decisions. In May 2019, the World Health Assembly Resolution WHA 72.8 on 'Improving the transparency of markets for medicines, vaccines, and other health products' was adopted and called on the Member States to improve transparency in different dimensions, such as on results and costs of clinical trials, suppliers' reports on sales and volumes and patent status information (WHA, 2019). Major attention of this WHA Resolution focuses on the commitment of Member States to work on publicly sharing so-called 'net prices' of medicines. This would include the disclosure of confidential discounts negotiated between public payers and industry for some, high-priced medicines in the course of so-called managed entry agreements.

The discussion on transparency, in particular of net prices, is highly controversial, and evidence on the impact of improved transparency on affordability and availability of medicines is mostly lacking, given the lack of the implementation of transparency policies (Webb et al., 2022). However, there have been initiatives to implement (parts of) the WHA 72.8 Resolution for instance a change in the Italian pricing and reimbursement legislation to request net prices of other countries and a legal change in France which introduced the obligation for pharmaceutical companies to disclose public

investment in research and development costs (Vogler, 2022a). Related research addressing managed entry agreements, assessment of clinical benefits and transparency policies will be hosted in Strand 2 of the PPRI Conference, entitled 'Strengthening the evidence base'.

Innovating pharmaceutical policies

While the COVID-19 pandemic has officially ended, the world will continue to be confronted with COVID-19 infections alongside many other infectious diseases. The 'silent pandemic' of antimicrobial resistence (AMR) is taking lives (Anderson et al., 2023). Non-communicable diseases continue to be the leading cause of disease burden globally (Murray et al., 2020). Climate change will impact the lives of millions of people and requires action in all policy areas (Romanello et al., 2022). These challenges add to the ones on access to medicines, as described above.

Thus, innovation that addresses public health priorities and sustainability is urgently needed. We certainly require pharmaceutical innovation to offer therapies in areas of unmet needs. In addition to break-through innovation, which has to be affordable to public health systems to ensure access and uptake, incremental pharmaceutical innovation also has its role but marginal benefits should be accordingly reflected in its price.

Innovation is not only a task for developers, but also for policymakers, as it is their responsibilty to design and implement appropriate pharmaceutical policies aligned with policy objectives. Policy implementation should consider learnings from previous experiences. In today's world, flexibility, creativity and innovation in policymaking is required to address current challenges. For more than a decade, there has been the call for fundamental reforms based on 'thinking out of the box' (Vogler et al., 2016). Although in practice debates have frequently been dominated by the refrain that proposed approaches (e.g. improving transparency) would not be feasible, the proof of concept in some areas, e.g., joint procurement of the COVID-19 vaccines in Europe (Vogler, Haasis, et al., 2021), provides needed evidence for progressive change.

We need to learn more from the implementation of existing innovative policies. They may include novel pricing or funding mechanisms (e.g., fair pricing concepts, subscription-based models for antimicrobials), elements to foster transparency, evidence and (cross-country) collaboration, and additional selection criteria in pricing, reimbursement or procurement decisions (e.g., award criteria of security of supply and environmental criteria in public tenders for medicines). In particular, innovation in policymaking requires overcoming existing silos: this implies, for instance, an improved understanding between regulatory agencies (responsible for marketing authorisation) and the downward actors such as HTA bodies, payers and pricing authorities, coordinated demand-driven approaches by public payers (supported, for example, by

horizon scanning to allow for preparedness) and patient-centered pharmaceutical and health systems. Lastly, multi-sectoral action, where non-health policy areas (e.g., local production, education) are aligned with public health issues, is another component of policymaking which is still innovative today but could and should be good practice in future.

Input presented in Strand 3 'Futureproofing pharmaceutical policies' aims to encourage innovative pharmaceutical policymaking. Together with the contributions in the plenary sessions and the two other strands of the 2024 PPRI Conference, they will trigger discussions on how to progress pharmaceutical policy globally to achieve sustainable access to affordable medicines. The multi-stakeholder audience attending the PPRI Conference in Vienna is an ideal forum for an open and evidenced-informed debate.

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