WHAT IS FAIR?

AIM POSITION PAPER ON FAIR MEDICINE PRICES

Debates on sustainability of medicine prices have come up the public health agenda over the past years. From a topic that mainly concerned low- and or middle-income countries that couldn't afford costly but vital medicines in the 1980s-1990s, it has become, under the joint pressure of strained public finances and rising medicines costs, a topic that many regional and global stakeholders have addressed more intensively since June 2016.

Ministers of Health of EU Member States agreed in June 2016 on Council conclusions underlining the challenges that very costly pharmaceutical products pose on health systems and recall the "consequences for pricing and reimbursement, the financial sustainability of health systems, their post-market surveillance and patient access and affordability" that new pharmaceutical products pose on health systems.[1] The European Commission closed a consultation on the relevance of introducing a common EU mechanism to conduct health technology assessments. Streamlined assessment procedures across the EU should help transparent decision-making as well as ensure that all member states set appropriate medicines price levels. The European Parliament adopted a Resolution on EU options for improving access to medicines.[2] The Resolution is not short of calls to control medicines prices increase, to take into account the need to ensure access to as many EU citizens as possible while rewarding the industry.

The key question is therefore the question of **price fairness**. On the one side, we have the industry, which has focused on the value of treatment, saying that a treatment's value, meaning that usefulness to society, justifies its price. Of course, value for money is important but how about other aspects? In a resource-scarce environment as the one that we have today, we should also take into account affordability, actual R&D costs, medical needs, the willingness to pay for medicines, or the added benefit over existing alternatives to name but a few other elements.

This paper was drafted as the World Health Organization (WHO) convened an advisory group on the topic of fair medicines pricing too.[3] The first meeting of the group took place end November 2016. The proceedings acknowledged that the pricing of essential medicines have become a matter of global concern, due to the excessive price asked for some useful medicines and the too-low profit margin offered for some generics.[4] The Organisation for Economic Co-operation and Development (OECD) also launched an online consultation on sustainable access to innovative therapies, asking respondents about current and future challenges with regards to access to medicines.[5] AIM welcomes these initiatives, their scope as well as preliminary observations and remarks where they exist and has drafted this paper to enunciate its position in this debate.

[1] http://www.consilium.europa.eu/press-releases-pdf/2016/6/47244642812_en.pdf
[2] http://www.europarl.europa.eu/sides/getDoc.do?pubRef=-//EP//NONSGML+TA+P8-TA-2017-0061+0+DOC+PDF+V0//EN
[3] http://www.who.int/medicines/access/fair_pricing/fpf_report/en/
[4] http://www.who.int/entity/medicines/access/fair_pricing/report_fair_pricing-forumIGmeeting.pdf?ua=1
[5] http://www.oecd.org/els/health-systems/sustainable-access-to-innovative-therapies-online-consultation.htm

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It is a no-brainer today to state that **medicines therapeutic performance should be the leading factor** to have a treatment included in care and reimbursement plans. When a treatment shows therapeutic added-value, it should be reflected in its price as well as reimbursement level. Still, too many treatments fail to show relevant outcomes at different stages of their lifecycle. AIM members reported many shortcomings they've faced during the market access phase: too fast marketing, with inconclusive evidence of benefit, due for instance to inexistent HTA procedures sometimes, or limited HTA-relevant clinical data.[6] Uncertainty about added-value is therefore a very important shortcoming. The trend towards reducing the regulatory approval phase, epitomised by the European Medicines Agency (EMA)'s adaptive pathways project should also lead to further caution in this regard.[7] AIM has already voiced in a position paper on adaptive pathways its concerns over this particular project, underlining the importance of running appropriate safety and efficacy studies via the pre-marketing procedure. AIM also expressed scepticism towards the ability of the different parties to fulfil the post-marketing requirements foreseen under the pilot project. In addition, when treatments are on the market, it is in practice also very hard to reassess their price or to de-reimburse them, which leads to an inefficient allocation of resources.[8]

We believe that medicines prices should also **reflect medical needs**. As a rule, medicines should help address societal and/or individual needs taking into account the therapeutic performance of alternatives. We recognise the usefulness of quality-adjusted life years (QALYs) measures, but note that they are of a concern when QALYs are the only element considered to make a decision on prices. Breakthrough therapies bring therapeutic advances that need and should be reflected in the final price. However, many products that only bring a marginal therapeutic benefit over existing products ("*me-toos*") reach the market and receive a high price, thus diverting investment decisions and budgetary resources away from therapeutically relevant areas. Therapies for rare diseases that bring substantial relief to patients must receive a higher price too, due to the relatively low target populations and budget impact, in comparison with the high individual burden. This is the initial rationale behind orphan drugs legislations. However, pricing of some of these therapies must be examined again, especially in the light of the "salami-slicing" practice, whereby manufacturers seek orphan designation and receive high compensation for multiple indications of the same disease, or of different diseases that, in total, cover a patient population greater than the standard orphan population size.

However, we also need to take into account that **manufacturers need to receive a reasonable retribution**, in order to support investment decisions. Such a reasonable retribution should be based on all the elements listed above: therapeutic value as well as added therapeutic benefit, but also other elements such as research, development and other manufacturing costs. It should also take into account factors specific to the regulatory framework and/or the specific market that the product is launched in, such as the duration of the expected market exclusivity or the size of the considered target population. Bigger target populations and longer market exclusivity times should drive prices down for standard, non-orphan treatments. Again, R&D costs must be looked at carefully for numerous reasons. First, public investment contributes to the overall research costs into the pharmaceutical sector and

[6] http://ec.europa.eu/smart-regulation/roadmaps/docs/2016_sante_144_health_technology_assessments_en.pdf
[7] http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000601.jsp
[8] https://www.aim-mutual.org/wp-content/uploads/2017/04/Adaptive-pathways-why-is-AIM-cautious.pdf

this needs to be reflected in the final price.[9] There is also an ongoing debate regarding what costs the final price should remunerate. This final price cannot cover only for the R&D price of the medicines that make it into the market and overlook the costs of developing unsuccessful treatments. At the same time, we must also not get into a situation where manufacturers make overly risky investment decisions expecting that their cost will be compensated later on through the price of successful treatments. Once on the market, a progressive extension of the initial patient population must also give governments and payers the legitimacy to reassess medicines prices.

Sustainability and inclusiveness of health systems is of course the last point to keep in mind. Regardless of effectiveness, paying high prices for medicines that will only benefit a few is not ideal nor wishable as it would contradict with many European societies' commitment to secure access to health for everyone. We however know that, ill- or bad health due to non-effective treatments can translate into an increased financial burden and waste. While governments want to maximise their population's health coverage within available resources, they also seek to ensure access to quality healthcare for all too. In relations to this, when it comes to specific diseases where very costly treatments have a significant budgetary impact but only slow down the disease course, we do not really know whether paying for a medicine is actually the best resource allocation. For these specific diseases, carers can sometimes prefer receiving an allowance so that they can fully dedicate to the specific person. A wider debate on the topic is therefore needed too.

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The framework in which prices are formed and evolve is actually as important as the final price levels. Indeed, achieving fair prices implies that a comprehensive framework is in place to help with the management of health technologies across their life-course. Such a framework should help make sure that certain number of tools and mechanisms are available to decision-makers so they can take the best-informed decision.

1. Increased collaboration between regulatory bodies and HTA bodies - At the time of the price-setting decision, governments must be able to assess the effectiveness of a treatment with the best information available on therapeutic added value. However, at this stage health technology assessment (HTA) is not used across the EU, nor are HTA bodies always presented with the information that is the most relevant to them. A clear effort should be made in this regard.
2. Increased transparency
    a. The European Parliament's report on access to medicines mentions a "high" level of public funding in pharmaceutical research but there is no indication of how high this level is, or of when in the development process this public funding takes place.[10] We need robust studies to understand the scale of public investment into biomedical research.

[9] https://curie.fr/sites/default/files/medias/documents/2017-09/Dossier%20de%20presse%20Observatoire%20cancer%202017.compressed_0.pdf
[10] http://www.europarl.europa.eu/sides/getDoc.do?pubRef=-//EP//NONSGML+TA+P8-TA-2017-0061+0+DOC+PDF+V0//EN

b. Another significant loophole is the lack of clarity regarding the actual prices that governments pay for pharmaceuticals, including discounts. The Euripid database is a relevant structure at a time when a significant number of member states use external reference pricing system. However, the European Parliament rightly noted in its report on access to medicines that Euripid falls short of providing reliable information regarding real prices, which therefore takes away the intended benefits for its users and therefore further adds to the lack of clarity on price levels.

3. Flexibility to reassess medicines prices - After marketing, strong, reliable and transparent mechanisms to reassess medicines price must be in place. Such a procedure is needed to review the price of a medicine based on the therapeutic value of new treatments entering the market, in order to free-up financial resources for newer more effective treatments. As a result, adequate technical resources, a suitable governance structure and enforcement system must be in place to support such a mechanism.
4. An adapted intellectual property protection framework - plays a key role in regulating prices in the pharmaceutical market too. Patent protection allows companies to recoup R&D investment before generics and biosimilars competition kick in and to support the development of treatments in therapeutic areas with high unmet needs, as it is for instance the case for orphan drugs. However, a favourable IP regime must not lead to a situation where companies decide to invest only in areas where they can ask a high price tag for their medicines. The European Commission's endeavours to reassess the definition of added therapeutic value in the frame of orphan designation is welcome in view of the current risk to "orphanise" the pharmaceutical treatment landscape.
5. A reflection on society's willingness to pay – so far, there has been no societal reflection and discussion over if the agreed QALYs levels reflect the actual society's willingness to pay for medicines for this additional life year. For specific diseases where very costly treatments only slow down the disease course, we do not really know whether paying for a medicine is actually better than giving an allowance to carers so that they can dedicate their time fully to the patient.
6. Alternative business models – The current market failures as well as the strain on public finances have led to a reflection about alternative medicines development models. The Belgian Federal Centre for Healthcare Expertise (KCE) produced a study on different scenarios for the development of medicines, ranging from public-private partnerships, to manufacturing medicines with public resources[11]. Such models are probably at this stage only indicative but at least help public authorities and health players with their reflection on future medicines development models.

[11] https://kce.fgov.be/sites/default/files/page_documents/KCE_271_Drug__Pricing_Report.pdf