Will fast-tracking new medicines improve affordability?

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european public health alliance

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Introduction

This briefing is a response to an article published on May 12 2016 by four influential European regulators including the leadership of the European Medical Agency (EMA), as well as the Director of the Dutch Medicines Agency,[1] addressing the issue of the affordability of new drugs. Among other issues, they outline what they can do about the high prices of medicines through initiatives such as the ongoing adaptive pathways pilot project (formerly known as adaptive licensing). Their article raises serious questions as regards to public health risks of such an approach and legal questions around the governance of pharmaceutical regulation.

Context

According to the EMA, adaptive pathways aims to improve timely access to new medicines "primarily in areas of high medical need." The eligible products would be put on the market earlier for small subsets of patients and their use would gradually be expanded based on additional data generated.[2] The public health community has voiced a series of concerns ranging from the questionable innovative value of these medicines to fears about patient safety and the affordability of these products. Therefore, it is essential for the public health community to see the evaluation of the pilot project, which must include the impact on affordability.

Public health concerns

Here are some preliminary remarks and questions on some of the points raised:

1. **Expanding the scope – Unmet medical need and beyond?**

"EMA is exploring whether a more flexible development, licensing and reimbursement approach called adaptive pathways may help companies stagger clinical development, costs, generate revenue earlier, and remove some risks from R&D without relaxing the criteria for determining products' benefit-risk profiles''

[1] Hans-Georg Eichler, M.D., Hugo Hurts, M.Sc., Karl Broich, M.D., Guido Rasi, M.D. Drug Regulation and Pricing — Can Regulators Influence Affordability? N Engl J Med 2016; 374:1807-1809.
[2] http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000601.jsp (accessed 19 May 2016).

Originally, adaptive pathways was strictly supposed to address "unmet medical needs",[3] by bringing eligible medicines faster and earlier to the market. In this new article, there is no reference to this scope at all. This is a fundamental question as one of the critics' key concerns[4] of the adaptive pathways school of thought has been that it seeks to introduce a new model applicable to all drug approvals[5] in Europe by turning the exception - early access - into the new rule. By neglecting to mention the scope restricted to "unmet medical needs." it implies that the fast track, light-regulation approach is indeed intended to become the de facto approach for approvals for all medicines. This would be a fundamental regulatory change through the back door, without comprehensive consultation of stakeholders or approval from policy-makers – and before the pilot project has even been formally evaluated in terms of patient safety or impact on access or affordability.

2. Primary objectives – Public health or competitiveness?

It is notable that the authors explicitly prioritize ***"helping companies stagger clinical development, costs, generate revenue earlier, and remove some risks from R&D".***

This subordinates the original primary purpose of the pilot project, to accelerate access, whilst ensuring patient safety. The credibility of the project and the evaluation of the results are at risk if cost reduction and revenue maximization of the pharmaceutical sector are being put ahead of the needs of patients, health systems and the protection of public health.

3. Is Affordability an objective or not?

Throughout the project, the European Commission as well as EMA officials emphasized that the issue of prices and pricing are outside their remit and therefore they did not aim to consider affordability within the adaptive pathways context. This latest article contradicts that emphasis, with affordability now being used to justify the shift in approach. The authors now assume that adaptive pathways could bring about affordable medicines. This raises questions of responsibility between the Agency, Commission and member states. But more fundamentally, there is a continued lack of evidence to support assumptions of improved affordability. "We expect that this kind of "life span" approach to generating evidence — with more targeted selection of trial participants, managed growth of the treatment-eligible population…will lower the threshold for financing drug development at a time when prices are coming under pressure" There is no evidence (yet) to substantiate the claim that the so-called "life span approach", including regulatory streamlining and the de facto lowering of evidentiary requirements[6] for the earlier and speedier approval of new drugs, will contribute to more affordable prices.

[3] http://www.ema.europa.eu/docs/en_GB/document_library/Other/2014/03/WC500163409.pdf (accessed 19 May 2016)
[4] HAI, ISDB, MiEF Joint Briefing paper "Adaptive licensing" or "adaptive pathways": Deregulation under the guise of earlier access 2015.
[5] Eichler H-G et al. "Adaptive Licensing: Taking the Next Step in the Evolution of Drug Approval" Clinical Pharmacology & Therapeutics 2012; 91 (3): 426-437.
[6] Windeler "Real world data – an asset for benefit assessments? How can registries and routine data contribute?" IQWiG Autumn Symposium 2015.

Affordability should indeed be one of the central criteria to judge whether the pilot has contributed to public health benefits. The European Commission should present an assessment backing this "expectation" with solid evidence, rather than working on the basis of an untested assumption. From the perspective of health services, payers and patients it is essential to test this assumption as some evidence points to fast track approaches leading to higher prices. One of the starting points of adaptive pathways is the emphasis on niches and subgroups of the general patient population.[7] The more flexible approach for orphan drugs for rare diseases arguably led to the abuse of the orphan drugs framework whereby "every drug wants to be an orphan".[8]

The emphasis on niches may result in the further orphanisation[9] of the pharmaceutical regulation with medicines ending up costing even more[10].

4. Continued absence of transparency

"The cost of conducting clinical trials drives R&D spending, and much of the elaborate super-structure involved needs to be re-assessed and could be pared down without harming participants."

This assertion is also unsubstantiated: The cost of medical R&D is an absolute black box with no transparency by manufacturers. It is inappropriate for senior regulators to unconditionally support this line of thought. This reiterates, once again, the imperative need for manufacturers to be forthcoming, as far as their investment in research and development of medicines is concerned.

Conclusion

It is welcome that regulators acknowledge the gravity of the problem of the high prices of medicines in Europe today and feel the need to take a stance. Framing adaptive pathways as a pilot project has prevented and impeded any political scrutiny. Following the May 12 publication in the NEJM, it becomes clearer than ever before that this new approach is not merely a technical discussion which can take place behind closed doors. It is a paradigm shift with massive political impact. Its implications cannot be discussed any longer in side meetings and in various expert fora (such as the European Commission's Expert Group on Safe and Timely Access to Medicines for Patients or the EMA) without political accountability and oversight.

Furthermore, stakeholders should be better involved in the project evaluation and transparency in the process needs to be dramatically improved. Having to rely on press clippings and journal articles in order to obtain information about a pivotal EMA project which started in Spring 2014, is conducted with taxpayers' money, and has far-reaching economic, political and public health consequences is truly alarming. It is high time the European Parliament, the Council, national medicines agencies, HTA

[7] Eichler H-G et al. "From Adaptive Licensing to Adaptive Pathways: Delivering a Flexible Life-Span Approach to Bring New Drugs to Patients" Clinical Pharmacology & Therapeutics 2015; 97 (3): 234–246.
[8] Gagnon MA "New drug pricing: does it make sense?" Prescrire Int 2015; 24 (162): 192-195
[9] Garjon. Orphan drugs: regulation and controversies. Drug and therapeutics bulletin of Navarre 2015; 23 (1)
[10] Daniel, Michael G. et al. The Orphan Drug Act: Restoring the Mission to Rare Diseases. American Journal of Clinical Oncology 2016; 39 (2): 210-213.

bodies, the payers and governments were properly involved, before any conclusions are reached about the success or otherwise of the pilot project.

About EPHA

EPHA (AISBL) is a change agent – Europe's leading NGO advocating for better health. We are a dynamic member-led organisation, made up of public health NGOs, patient groups, health professionals, and disease groups working together to improve health and strengthen the voice of public health in Europe. EPHA is a member of, among others, the Social Platform, the Health and Environment Alliance (HEAL), the EU Civil Society Contact Group and the Better Regulation Watchdog.
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