European Federation of Pharmaceutical Industries and Associations

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Public Consultation on the preliminary opinion on *Access to health services in the European Union* by the Expert Panel on effective ways of investing in health

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✱ Briefing

Executive Summary

The following is the response from the European Federation of Pharmaceutical Industries and Associations (EFPIA) to the European Commission and its Expert Panel.

EFPIA welcomes the debate on access to health services, but it is a debate that has to go beyond simply the cost of individual medicines. EFPIA's aim is not to promote spending on health or medicines at all costs, but to work with healthcare systems to ensure that spending on medicines, and spending on healthcare as a whole is informed by patient outcomes and based on an understanding of the broader societal, economic and health benefits of biomedical research and innovative medicines. Medicines are part of the solution in addressing the real challenges faced by European healthcare systems.

Europe needs outcomes driven, sustainable models of healthcare delivery that focus on improving patients' health in a holistic and evidence-based way. Europe needs systems that allocate resources towards those interventions that deliver the best possible outcomes and away from those that don't, these systems improve quality. This is often less expensive in the long-term and thus more sustainable than the current transaction-oriented approach to healthcare.

Questioning the IP incentive system, which sustains pharmaceutical innovation, is unlikely to provide a sustainable solution to access to medicines and should be measured along the cost and the health impact of not having such innovative medicines.

Public Consultation on the preliminary opinion on *Access to health services in the European Union* by the Expert Panel on effective ways of investing in health

The following is the response from the European Federation of Pharmaceutical Industries and Associations (EFPIA) to the European Commission and its Expert Panel.

The opinion provides interesting evidence around access to health in Europe. The fact that nearly 18 Million people living in Europe, i.e. 3.6%, face unmet health need is concerning. The main question "How do limitations and variations in access to health care affect EU health systems and the broader economy?" is addressed by many aspects in subsequent eight chapters. Nevertheless, the report goes only half way as it remains restricted to the limitations and variations in terms of access to healthcare; it does only provide little context on the impact on EU health systems and the broader economy.

EFPIA welcomes the debate on access to health services, but it is a debate that has to go beyond simply the cost of individual medicines. **EFPIA's aim is not to promote spending on health or medicines at all costs, but to work with healthcare systems to ensure that spending on medicines, spending on healthcare as whole is informed by patient outcomes and based on an understanding of the broader societal, economic and health benefits of biomedical research and innovative medicines.** Medicines are part of the solution in addressing the real challenges faced by European healthcare systems.

The reality is that medicines are not the main driver of rising healthcare costs. In fact, spending on medicines has risen far slower than overall healthcare spending. As a percentage of GDP, spending on medicines has actually fallen in Europe over the last decade. Averaging around 16.9% total spending on healthcare, the medicines bill in Europe is currently under control and does not pose a threat to the sustainability of healthcare financing in Europe. These facts should be coupled to the pharmaceutical industry continuing to drive a positive trade balance for Europe, employing over 700,000 employees across the EU and spending more of its revenue on research and development than any other industry.

Somehow the debate often fails to recognize that innovative medicines have transformed the way health systems can treat numerous diseases and how these diseases impact on the lives of patients, their families and carers across Europe. There are numerous examples; between 2004 and 2013, the number of death among aids cases in Europe decreased by 75%[1], since the 1990, the cancer death rate has fallen by nearly 22 percent and new hepatitis C therapies have cure rates of up to 95 percent"[2]

[1] HIV/AIDS surveillance in Europe 2013, WHO Regional Office for Europe & European Centre for Disease Prevention and Control (ECDC).

[2] PhRMA (2014), 25 years of Progress against Hepatitis C and PhRMA (2015), 2015 profile available at: http://www.phrma.org/sites/default/files/pdf/chartpack-2015.pdf.


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Prices set for innovative medicines may be discussed and debated, but the patent system should not be called into question as part of this discussion. This debate should not confuse whether there is a patentable invention (which is determined by internationally accepted standards) with the question of the price or value of a marketed product. Patents drive innovation and an uninterrupted flow of innovative medicines with consequent benefits to patients and wider society. EFPIA is therefore concerned that the opinion proposes measures such as withdrawing patent protection from innovative companies (l. 2611) or using mandatory licences (l. 2792). **While EFPIA supports quick and effective transition to off-patent markets, this transition should not be accelerated by undermining patents to address pricing issues.** Such a policy would not only be at odds with the objectives of promoting investments in health and in R&D but would also greatly jeopardise the EU position as a leader in life sciences and innovation. Indeed it would call into question the EU's commitment to an innovation-based economy more generally.

All too often the focus of the debate has centered on the price of individual medicines. However, we know overall medicine spending has fallen as a percentage of GDP. **Particularly over the past 6-7 years, cost containment efforts have primarily focused on medicines rather than other healthcare segments or interventions.** This reflects the pressure to make short-term savings at the expense of structural reforms or long term outcomes driven, sustainable healthcare delivery.

Thought leaders recognize that patients and healthcare systems would be better served by a more holistic dialogue, encompassing the management of healthcare budgets and managing the introduction of new medicines. **Europe needs outcomes driven, sustainable models of healthcare delivery that focus on improving patients' health in a holistic and evidence-based way.** We need systems that allocate resources towards those interventions that deliver the best possible outcomes and away from those that don't, these systems improve quality. This is often less expensive in the long-term and thus more sustainable than the current transaction-oriented approach to healthcare.

General comments on the preliminary opinion

The opinion would benefit from a **clearer structure by prioritizing the reasons for unmet need** and lack of access. As stated on page 35, line 1223f, "lack of affordability is the single most important factor behind self-reported unmet need in EU countries". Since the latter is linked to the way healthcare is provided, e.g. health coverage, the health system perspective would have deserved greater attention in the overall policy recommendation. As stated in Chapter 2 lack of access due to financial barriers depends on what extent governments invest in healthcare and the coverage within the respective health system (as also shown in Fig. 2.1, Relationship between out-of-pocket payments and public spending on health, EU28, 2013). **To what extent health budgets of Member States are interlinked with access and health outcomes is only partly addressed**. In addition, the assessment of the impact of access to health on the broader economy – the initial intention of the expert question – falls short; it is unfortunately only briefly discussed on page 12, lines 399ff. with mainly referencing to existing sources.

As underpinned by the opinion health is an important contributor to wealth [line 399: "better health drives economic growth, greater labor force participation and higher productivity"]; as such it also contributes to Europe's economy and, ultimately, competitiveness. **More detailed reflections about the**


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connection between access, health and competitiveness may be also useful for positioning the opinion among a broader audience and may help to gather stronger support for investment in and financing of health.

EFPIA believes that the perspective of the authors on **transparency of prices** (see for example line 2524) **is flawed and does not take into account the current context**: it assumes that greater price transparency will lead to greater savings and therefore a more optimal outcome for the healthcare systems, without factoring its negative impact in terms of patient access and incentives for innovation. For instance companies may model the impact of individual pricing decisions on their revenue in Europe and adapt country pricing decisions accordingly. They may also decide not to launch in countries where the price would adversely affect overall revenue in other countries (also outside Europe) or launch at a higher price predicting quicker erosion. Other potential responses would be to seek reimbursement in smaller patient populations to maintain higher prices and avoid EU-wide price erosion. Companies have already been adapting their behaviour over the last 5-10 years, which is proven by the smaller variation in the prices of newly launched products. The authors have not taken into account any temporal element and therefore did not explore how the trends in European pricing have changed over time.

Prices set for innovative medicines may be discussed and debated, but the patent system should not be called into question for these. Most importantly, this debate should not question or deny the value of an invention and the fact that, as such, it does deserve a patent from the patent system, which operates according to internationally agreed requirements and only with a view of rewarding and fuelling innovation. Further, this debate should not ignore or deny the significant value to patients and to the wider society brought by this invention and an uninterrupted flow of innovative medicines. EFPIA is therefore concerned that the opinion proposes measures such as withdrawing patent protection from innovative companies (l. 2611) or using mandatory licences (l. 2792). **While EFPIA supports quick and effective transition to off-patent markets, this transition should not be forced to address pricing issues and at the detriment of innovation.** Such a policy would not only be at odds with the objectives of promoting investments in health and in R&D but would also greatly jeopardise the EU position as a leader in life sciences and innovation.

EFPIA questions **the opinion of the Expert Panel that there is a conflict of interest on the funding model of the European Medicines Agency (lines 2578ff) and suggests that the term "conflict of interest" is inappropriately used in the document within this context**. The rules relating to the Agency's fees are governed by the fee Regulation (Council Regulation (EC) No 297/95) and its implementing rules, as well as the pharmacovigilance fee Regulation (Regulation (EU) No 658/2014). The funding model is regarded to be transparent and has been subject to formal legislative procedure through European Parliament, Council and Commission. Additionally, national Medicines Agencies have their own requirements for fees, which they charge for their services conducted on the national level and following the same primary objective to provide protection to the public health.


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Recommendations

In order to improve the opinion, EFPIA recommends additional work to be done. This would be of value for further assessing the scope of some policy recommendations, and might lead to opposite recommendations than the ones currently highlighted in the report.

EFPIA suggests to the authors:

- To prioritize the issues of access and potential solutions by **explaining on what other factors access to health services depends** (e.g. health system coverage, investments in healthcare infrastructure, access to specialists, early diagnosis and screening, restrictive clinical guidelines, delays in some countries for products to enter the market after marketing authorization etc.) and to what extent health budgets of Member States are interlinked with access and health outcomes. Refocus Chapter 6 on medicines and medical devices and to include recent findings on pricing and reimbursement policies such as External Reference Pricing and Parallel Trade

- To mention the current thinking on **innovative pricing approaches** of linking price with outcome performance, adaptive pathways and use of real-world evidence. This may not be within the scope of this opinion, but a proper reflection is needed about mechanisms for the new types of medicines coming onto the market. In other words, it is not clear how this project connects with other policy areas at EU level, e.g. adaptive pathways and dynamic pricing, EU registries, etc.

- To undertake further research **to understand the causes of drug shortages and work towards their prevention**. Addressing the problem of shortages meaningfully with the aim of improving patient access to medicines will require the involvement and commitment of all relevant supply chain stakeholders. Instead of fuelling market distortions leaving patients in lower income, lower price countries, worse off, the objective must be to move away from importing low priced products from other countries to having an affordable price in each market based on objective criteria. EFPIA encourages national authorities, potentially in a EU forum, to consider practical ways of increasing transparency in the supply chain and to foster greater solidarity among Member States to reduce disruptions in the supply chain.

- As the authors mention 'the absence of information on prices' (2518) EFPIA recalls that the Delegated Act to be released by the European Commission in the context of the Falsified Medicines Directive (Directive 2011/62/EU) provides for the establishment of an interoperable repositories or database systems containing unique identifiers for prescription medicines. Given the Directive's legal basis which grants the right to Member States to use the system for other purposes e.g. reimbursement, pharmaco-vigilance and pharmaco-epidemiology purposes, research could be undertaken to assess to what extent the repositories' system could be extended to include price information in light of the Transparency Directive requirements.


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Comments on the preliminary opinion by chapter

Abstract

As underpinned by the opinion health is an important contributor to wealth [line 399: "better health drives economic growth, greater labor force participation and higher productivity"]; as such it also contributes to Europe's economy and, ultimately, competitiveness. More detailed reflections about the connection between access, health and competitiveness may be also useful for positioning the report among a broader audience and may help to gather stronger support for investment in and financing of health.

Terms of reference

The opinion recognizes the importance of socio-economic factors such as income, education, employment etc. (Chapter 1 and 2) and the role of health literacy (Chapter 7). **The former shows that health does not only depend on health policy but that health should also be an element in other policies.** The latter – the role of health literacy – explains the complex interaction between users, providers, the health system and other areas of public policy: "Access barriers are rarely attributable simply to the user, the provider or the health system alone but rather to the lack of alignment between these different levels. Health literacy, for example, is the result of a mismatch between a person's ability to understand health information and the provider or health system response (Parker and Ratzan 2010)." Tackling socio-economic issues and improving better interaction requires broader policy action "beyond the health system" [line 578f.], i.e. "intersectoral action [line 726f.] towards "health in all policies". Although this may be out of scope of the report, further considerations in this direction would have been interesting.

Given the terms of reference, it is surprising that **'unmet needs' is not very well defined in the opinion**. The authors speak quite often about antimicrobial resistance, but there are also other areas as defined by the WHO's *Priority Medicines Report*. On a different note, **there is also no clear explanation of what the opinion tries to obtain**. The proposed recommendations at the end of the sections are either conceptual or very concrete. Especially in section 6 of the opinion "Quality of medicines' the opinion appears a bit biased towards focussing on pharmaceutical pricing. The recommendations on pricing seem the most 'actionable', suggesting the short-term nature gains of that section, whilst in other areas the authors stick to a more high level approach.

Section "An introduction to access to health services in the European Union"

Healthcare workers remain a key trusted source of information for citizens and patients (Yaqub et al. 2014[3]) and should therefore act as drivers towards vaccination and other preventative measures in the interest of public health. Countries that have appropriate incentive systems in place (i.e. NL, UK) are shown to be able to remove some of the existing barriers**. EFPIA suggest further work on the provider-level barriers (lines 661ff).**

[3] http://ac.els-cdn.com/S0277953614002421/1-s2.0-S0277953614002421-main.pdf?_tid=4c0cefac-7955-11e5-ada6-00000aacb35e&acdnat=1445584476_48eb2498d3ed732d289e170cc73f5929


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EFPIA agrees that one of the major obstacles for access to health is financial barriers. In the abstract [line 143] but also in the introduction [lines 309ff.] it is mentioned that financial barriers to access are "the largest single driver of unmet need in the European Union". However, this statement is often presented in isolation. **It would require a more holistic analysis in order to explain what other factors access to health services depends.** As an example, access to treatments for patients suffering from Multiple Sclerosis varies from 13% to 69% depending on where you live in Europe. The causal factors behind such inequalities are many and also relate to delays in diagnosis and access to neurologists specialized in MS (e.g. in the UK where there is only 1 specialised neurologist per 100.000 inhabitants; compared to 3 in Romania and 11 in Austria), how clinical guidelines are used in practice (there are more and more restrictive guidelines notably in low income countries), the existence of reimbursement barriers (using Health Technology Assessments (HTA), a number of countries have restricted the use of the innovative medicines by reimbursing only certain patients who meet strict eligibility criteria), or arbitrary administrative processes which, combined with national and regional variance in HTA assessment, result in significant delays in some countries for products to enter the market after marketing authorisation[4].

It would also require further explanation as it could be easily misunderstood that healthcare is unaffordable as such. As the report states financial barriers are multi-dimensional as e.g. the Figure in the abstract but also Figures I.2-5 show: a major determinant for lack of access seems to be the socio-economic status (income, education, work status etc.). **The respective statement on "financial barriers" should be further qualified in the Abstract, the introduction and the conclusions**.
Tackling socio-economic issues requires broader policy action "beyond the health system" [line 578f.], i.e. "intersectoral action [line 726f.]. Although this may be out of scope of the opinion, further considerations would have been interesting.

The considerations raised in the opinion (lines 813ff) by the panel are certainly of important relevance for healthcare systems today. **A particular area that deserves mention and more attention and investment is specifically the area of 'prevention'**. It is known from the latest OECD figures available that spending on health prevention averages about 2.7% of OECD healthcare budgets only. Moreover spending on prevention is erratic across years and is one of the first areas to suffer from cuts at times of crisis. This is considered to be not sustainable. An adequate level of prevention must be ensured, and this also should be commensurate to the public health need in the relevant Member States. Vaccination represents only a small fraction of this budget (an estimated 0.6 of healthcare budgets), with room for using funds more efficiently as well as increasing budget allocations to secure public health. Also, the availability of data on prevention spending is scarce and there is room for improving the assessment of public investment in this regard including on key performance indicators, establishing the appropriate information system.

[4] Access to medicines for multiple sclerosis: Challenges and opportunities. Charles River Associates (2014). http://www.crai.be/sites/default/files/publications/CRA-Biogen-Access-to-MS-Treatment-Final-Report.pdf


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Section 3 Services are relevant, appropriate and cost-effective

EFPIA agrees that resources required to deliver relevant, appropriate and cost-effective health services – financial and human resources, facilities and interventions – are as closely matched to need as possible. It would be helpful to have more context on these three criteria since this will be critical in determining other questions around coverage and reimbursement – the latter being key for access see lines 1746ff):

- In particular, **it would be helpful to have more context on "relevance":** The explanation "the services available broadly correspond with the health needs of the population" [line 1752] is rather vague, and there are many variations possible up to the other extreme "of making everything available to everyone at all times" [line 127, Abstract]. "Relevance" may depend on preferences and social conventions, and differ from country to country. In addition, the term "cost-effectiveness" is defined rather narrow: "Services are defined and delivered in relation to cost-effectiveness, meaning that benefits should outweigh costs and, where alternatives are available, the most cost-effective option is chosen" [lines 1761ff.].
- Lines 1761ff., cost-effectiveness is defined as "the point at which the minimum amount of input (and therefore cost) is used to achieve a given output"[5], not necessarily "benefits outweighing costs". It is usually also defined as the ratio of the cost of the intervention to a relevant measure of its effect. "**Cost-effectiveness" is a relative term** and assesses the incremental effect and cost versus a comparator. Whether an intervention is cost-effective, therefore, depends on a threshold which depends on various factors and social convention.

The comments on the following charts may **incomplete**:

- Line 1797, Fig. 3.1: it has also to be explicitly noted that nearly all countries reduced hospital admissions for uncontrolled diabetes which means that improvements have been made in terms of cost-effectiveness. Besides these figures, it would be relevant to include figures on where problems lie and the pre-set vaccination coverage rates are not met for key public health areas of intervention. This is the case for influenza. The ECDC Technical Report published in 2014 as Annex to the Commission SWD 2014 (8) final shows how far Member States are from reaching the 75% vaccination coverage target for the elderly, and the issues in monitoring coverage for key target groups that represent a significant burden for healthcare systems such as chronic patients. Also vaccination on measles is lagging behind public health objectives; this raises clear concerns, with recurring outbreaks that are costly albeit preventable (ECDC, 2015[6]).
- Line 1808, Fig. 3.2: While it is correct that "in 2013, the share of children immunised against diphtheria, tetanus and pertussis ranged from 83% to 99%" it has also to be noted that *only one* country had a rate of 83%, and that in 2013, all the other countries were above 90% with nearly the half close to 99%.

[5] E.g. U.S. National Library of Medicine; https://www.nlm.nih.gov/nichsr/edu/healthecon/glossary.html (accessed: 19/10/2015)

[6] http://ecdc.europa.eu/en/press/news/_layouts/forms/News_DispForm.aspx?ID=1273&List=8db7286c-fe2d-476c-9133-18ff4cb1b568&Source=http%3A%2F%2Fecdc%2Eeuropa%2Eeu%2Fen%2Fhealthtopics%2Fmeasles%2FPages%2Findex%2Easpx


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Lines 1827ff: the authors recommend to "Put in place systematic priority-setting processes to enable HTA-informed, cost-effective coverage decisions for both new and existing technologies." **EFPIA would supports evidence-based decision-making, and therefore HTA.** Contrary to what the opinion suggests (lines 1828-1829 'altough it tends to be the exception rather then the norm') most Member States use HTA to support their coverage decisions today, as confirmed by an analysis conducted by the European Network on HTA (EUnetHTA) in 2011.[7]

A lot of focus on HTA is linked to disinvestment (lines 1836ff, "HTA focuses on new technologies rather than on disinvestment: To date, only a handful of EU countries systematically uses HTA for disinvestment (de-listing of existing benefits)"). As mentioned above, **HTA is a scientific tool which provides evidence, which in turn can be used to support priority setting, allocate resources efficiently, but not to actually take the decision**. However HTA does not actually take the decision. Disinvestment takes political will, which has little to do with HTA.

Lines 1896ff on **"Over medicalization": the paragraph can be misleading as it seems to stipulate a legal context which is not existing in Europe**, e.g. "Some domains of daily life are increasingly subjected to medical definition and jurisdiction, often as a result of 'disease mongering', a process in which interested parties create public awareness of and demand for specific treatments *through direct to consumer advertising, use of the news media and other strategies* (Moynihan and Cassels 2005)" [lines 1896ff., italic by the commentator]. In contrast to the US, direct-to-consumer advertising (DTC) is forbidden for prescription medicines, exactly for the reason of unnecessary promotion and its potential negative impact on public health budgets. DTC may be done for non-prescription medicines; however, such non-prescription medicines do usually have no impact on the publically funded health budget. Since the chapter is about "relevant, appropriate and cost-effective" spending in the public sector; therefore, this paragraph should either be deleted or explain to what extent it concerns "publicly financed benefits" [1705].

Section 4 Well-equipped facilities are within easy reach

EFPIA agrees that geography and infrastructure can be real barriers for access. **Modern technologies in the field of E-health and M-health offer many solutions, which do not only improve access but are very often also very cost-effective.**[8] To give one example in the area of availability of healthcare professionals: There are 8000 times more mobile phone customers worldwide than healthcare professionals for mental health; in low-income countries the ratio is even 1:30'000. Jones et al. show projects of successful use of mHealth applications that improves access to mental health services where healthcare professionals are scarce.[9]

[7] Except for the USA, all jurisdictions included perform evaluations that include a comparative analysis of efficacy and/or effectiveness of pharmaceutical(s) in comparison to alternative(s) to feed national reimbursement decisions on pharmaceuticals

[8] See e.g. Klasnja, Predrag; Pratt, Wanda (2012), Healthcare in the pocket: Mapping the space of mobile-phone health interventions. In: Journal of Biomedical Informatics 45 / 2012, S. 184-198; or: European Commission (2014),


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Section 6 Quality medicines and medical devices are available at fair prices

Chapter 6 Summary

The way chapter 6 addresses access to medicines and medical devices has some shortcomings; **except for a general statement, there is no analysis or evidence on access to medicines presented**. In nearly all European countries, medicines – as long as they have undergone P&R assessments – are covered by public health insurance, in particular so-called "high-price medicines". Nor does the opinion take into account recent findings of WHO, OECD and other institutions, which show that the lack of access is also a result of certain pricing and reimbursement policies in Europe. Finally, in some parts the opinion seems to mix access to medicines with pricing, e.g. when discussing R&D costs, pricing and "fair prices". **A more comprehensive discussion of value, price and health outcomes would be needed to cover this question appropriately.**

From a broader perspective, retail medicines expenditure accounts for approximately 16% of total healthcare spending; focusing on the most recent ten years, retail pharmaceutical spending across the OECD has, on average, grown more slowly than overall health spending.[10] Since 2009 pharmaceutical expenditure decreased by 2.2% while growth in total expenditure per capita decreased by 0.8%[11]. In addition, nominal medicine prices decreased in Europe by 24% between 2000 and 2013, in contrast to a 30% rise in consumer prices.[12] The opinion does not provide this context but seems rather to suggest that "the high price of many medicines is becoming an increasing problem for health systems in EU countries, threatening fiscal sustainability" [line 2503].

The report seems to lose the focus when discussing R&D costs, pricing and "fair prices". Access to high-cost medicines is primarily a question of coverage and healthcare budgets; other services in healthcare such as CT scanning or specific surgery like liver transplantations are also extremely costly interventions and depend on coverage. A more comprehensive discussion of value, price and health outcomes would be needed to cover the question about pharmaceutical pricing appropriately.

Last but not least, the report's focus on medicines prices falls short of recognizing that patient **safety should be a paramount priority, ahead of any economic considerations**. The practices of promoting off-label use of medicines for economic reasons, introduced in Italy and France, not only undermines patient safety, by exposing a patient to a medicine that has not been tested and approved for treatment of the patient's condition, but also put health outcomes at risk.

Green Paper on Mobile Health Services („mHealth"); COM(2014) 219 final; http://ec.europa.eu/digital-agenda/en/news/green-paper-mobile-health-mhealth

[9] Jones, Sarah P et al. 82014), How Google's 'Ten Things We Know To Be True' could Guide the Development Of Mental Health Mobile Apps. In: Health Affairs 33; 9 / 2014, S. 1603-1611; see also: Roediger A (2015), mHealth – on the way to health literacy 2.0 (German); in: swiss academics reports, Gesundheitskompetenz in der Schweiz – Stand und Perspektiven; 2015; 10(4); p. 77-78

[10] OECD (2015), Pharmaceutical expenditure and policies: Past trends and future challenges; DELSA/HEA(2015)6; p. 14

[11] OECD health statistics database and Europe at glance 2014 (accessed via elibrary in April 2015)

[12] EFPIA, "Health & Growth: sustainable healthcare systems" evidence compendium 2015.


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Promotion of economically-driven off-label use constitutes a significant disincentive for innovation by creating market uncertainty for companies, who will be less willing to undergo the costly, risky and time-consuming research and development process. EFPIA is concerned that the report does not acknowledge the risks for patient safety and for innovation environment stemming from economically-driven off-label use. EFPIA recommends that the report calls to effectively stop such practices.

6.1. Medicines

Line 2547ff: "People living in EU countries have publicly financed entitlement to a wide range of medicines. And yet there are thousands of people who cannot access the medicines they need and there are many conditions for which effective medicines are lacking." Unfortunately, **no further data or evidence is presented to understand the basis of this statement**.

The **issue of access delays** has been shown by Glynn (2014): "Lower income Eastern and Southern European countries tend to face longer delays than their Western and Northern European counterparts. At the extremes, Portugal had to wait an average of 46 months for new oncology drugs after they were released elsewhere in Europe. Switzerland (not an EU member) and the Netherlands had to wait just 5 months. For diabetes drugs, Croatia had the longest delay at 37 months, while Switzerland again had to shortest delay of just one month and five wealthy EU Member States waited only about two months."[13]

Reasons for the lack of access are manifold: With regard to External Reference Pricing (ERP) the WHO report on Access to Medicines in Europe gives further background on potential access issues resulting from this policy: "While ERP may help contain costs by reducing prices, critics are concerned about arbitration of the targeting price, launch delays and the lack of incentives for innovation. Sweden, the United Kingdom and – until recently – Germany are characterized by relatively free pricing mechanisms for pharmaceuticals. This, coupled with their strong local pharmaceutical industries, has often led them to be the first to adopt pharmaceutical innovation (15), although there have been concerns with the rate of adoption of new medicines in the United Kingdom, including new cancer medicines (see Table 13 in section 6.1). Further, in order to hinder low-price spillover through ERP, products are often launched in higher-priced EU markets, which can lead to launch delays and high launch prices in lower-priced EU markets such as Portugal and Spain (15; 16) or no launch at all in less wealthy countries."[14]

Lines 2575ff, see also further line 2837: **it remains unclear how this paragraph about the EMA relates to the question of access. EFPIA suggests that the term "conflict of interest" is taken out since the term is inappropriately used in the document within this context**. It seems rather to stipulate that EMA decisions were biased towards the industry with regard to marketing authorization because industry has to pay application fees. Such fees are quite common in other areas of public services (e.g. applying for a car license), and such claims would require further evidence and also explanation to what extent this relates to the main topic of the report, i.e. access to health services. On top of that, there are already measures in place to ensure the independency of the regulator from the industry:

[13] Glynn D (2013), Europe Economics (2013), External Reference Pricing; http://www.europe-economics.com/publications/15/publications.htm

[14] WHO Europe (2015), Access to new medicines in Europe: technical review of policy initiatives and opportunities for collaboration and research; p. 60


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- For example, the European Medicines Agency has a policy on handling of declaration of interests for its scientific experts, including committee members. The current policy was endorsed by the Agency's Management Board in March 2014 and entered into force on 30 January 2015.
- The European Medicines Agency takes a proactive approach to identifying cases where the potential involvement of an expert in Agency activities needs to be restricted or excluded due to interests in the pharmaceutical industry, and to searching for alternative experts where necessary.
- The EMA screens each expert's declaration of interests to decide whether or not to include him or her as a member of a committee, working party or other group. It assigns each declaration of interest an interest level based on whether the expert has any interests, and whether these are direct or indirect.

Lines 2581ff, prices: the Council has stated that new high-price medicines were of concern; this was also shared by OECD and the WHO Coordination Center. Nevertheless, the concern of the Council is rather about the budget impact; as stated above, the challenge is less affordability for the individual patient but finding solutions that ensure sustainable healthcare but also incentives for innovation.

Line 2590ff: the patent system often gets criticised for the wrong reasons and EFPIA would like to recall that pricing mechanisms and the patent system are distinct and separate entities. Prices are set up according to specific criteria and in negotiation with the relevant authorities and buyers at the Member States' level. The patent system has no bearing on how prices are fixed nor at which level they are fixed. As a consequence, it is inappropriate to blame or threaten the incentive system or refer to "patent misuse" to criticise prices or the lack of detailed knowledge of costs and prices, when none of these are objectives of or consequences of the patent system. Most importantly, this debate should not question or deny the value of an invention and the fact that, as such, it does deserve a patent from the patent system, which operates according to internationally agreed requirements and only with a view of rewarding and fuelling innovation. EFPIA is therefore concerned that the Expert Panel proposes measures such as withdrawing patent protection from innovative companies.

Line 2590ff: **the patent system often gets criticised for the wrong reasons and EFPIA would like to emphasise that pricing mechanisms and the patent system are distinct and separate entities.** Patent applications are filed much earlier in the R&D process, many years before a product even exists, is authorised or given a price, provided there is a product at all. Prices are set up according to specific criteria and in negotiation with the relevant authorities and buyers at the Member States' level. The patent system has no bearing on how prices are fixed nor at which level they are fixed. As a consequence, it is inappropriate to blame or threaten the incentive system or refer to "patent misuse" to criticise prices or the lack of detailed knowledge of costs and prices, when none of these are objectives of the patent system. This debate should not confuse whether there is a patentable invention (which is determined by internationally accepted standards) with the question of the price or value of a marketed product. Patents drive innovation and an uninterrupted flow of innovative medicines with consequent benefits to patients and wider society.


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EFPIA is therefore concerned that the Expert Panel proposes measures such as withdrawing patent protection from innovative companies to regulate prices. Such measures would be a strong deterrent to R&D investments and innovation.

Lines 2601ff: what evidence is there that prices are based on "what the market will bear"? Authorities set the rules of pricing and reimbursement in national legislation that pharmaceutical companies must follow; tools to reduce the effective prices of medicines that authorities are using include e.g. mandatory rebates or price cuts. What is meant by the statement "medicines have become financial products"? Medicines are not tradable investments.

Line 2640: to say “Member States approved reduction of prices” needs to be clarified. As Leopold et al. e.g. stated in recent research: “Economically stable countries implemented two to seven policy changes each, whereas less stable countries implemented 10 to 22 each. Of the 88 policy changes identified, 33 occurred in 2010 and 40 in 2011. They involved changing out-of-pocket payments for patients in 16 cases, price mark-up schemes in 13 and price cuts in 11.”[15] Most often, such **changes in policy were imposed by the countries and not “approved”**.

Line 2641: to say “In some cases there have been used reference prices” is understating: **26 out of 28 Member States of the European Union apply External Reference Pricing**.[16]

Lines 2648ff: pricing for the majority of the administered vaccines does not represent a barrier to access. In the case of routine vaccination **the major issue is acceptance and uptake of currently available vaccines** included in the national immunization programs. In these regards, joint procurement agreements would not help addressing the problems faced and securing appropriate protection of EU citizen’s health. Implementing the joint procurement in such cases risks hampering the sustainability of vaccine supply and creating concentration of demand, which could in turn jeopardize the ability to respond to the Member States needs.

Lines 2684: the following statement should be clarified and/or referenced: “Millions of people in EU cannot afford to pay medicines that they need. Medicines that are not publicly financed and reimbursed, or that have too-high user charges are not accessible for patients because of their economic situation.” **In Europe, most of the prescription medicines that have undergone P&R assessment are reimbursed by the respective Member State**. Nevertheless, there are differences in terms of making medicines available.

Lines 2689ff: it remains unclear how the paragraphs on “prescribing”, “use of medicines” and “disinvestment” relate to the core topic of the report, i.e. access. While we agree that these are important **topics they seem to be more relevant in the context of healthcare efficiency**.

[15] Leopold C et al. (2014), Effect of the economic recession on pharmaceutical policy and medicine sales in eight European countries; Bulletin of the World Health Organization 9(92):630-40

[16] Gesundheit Österreich and Sogeti (2015), Study on enhanced cross-country coordination in the area of pharmaceutical product pricing. Commissioned by the European Commission under the third Health Programme (2014-2020) (Draft), p. 16


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Line 2698: patient concern for the safety and efficacy of generics and, in particular, **biosimilars** is a reality in some cases. However, as it stands this statement does not provide adequate context to the issue it is eluding to (patient education) nor does it link to the issue of adherence which is discussed in the rest of the paragraph. **EFPIA would suggest separating these topics and providing more detail on the issue of creating sustainable and competitive off-patent markets,** which includes the production of accessible information for patients on the safety and efficacy of generics/biosimilars.

Line 2711, Box 6.1 Antibotics: the report states that "There have been almost no new antibiotics since the 1970s and there are none at all in the current development pipeline." This is in contrast to current development projects: "There are at least 53 systemic antibiotic NCEs in clinical development, of which 13 have reached Phase III testing."[17] The PEW Charitable Trust mentions in July 2015 that as of March 2015 of the 36 antibiotics in development, eight were in phase 1 clinical trials, 20 in phase 2, and eight in phase 3.[18]

Lines 2757ff., Box 5.3 Hepatitis C**: the report does not reflect the variation of prices** of Hepatitis C medicines such as Sofosbuvir. In addition, the following statement is too general and misleading: "This means that it is, in effect, unaffordable for most of those affected in several European countries. Generic versions of Sofosbuvir can be produced for under $300 per course (Hill et al 2014)"; the report seems to assume that Sofosbuvir was not covered by public health insurance which was not the case; in addition, many payers and governments negotiated lower treatment prices. It is also concerning that the opinion would endorse a practice of producing generic versions of an innovative medicine whilst patents are still valid in Europe. We repeat points made earlier about the need to keep questions about patents and prices separate and that such a practice would be a strong deterrent to innovation.

Policy responses

Line 2786: "Prices should cover the costs of R&D and production and allow for a reasonable profit…". The opinion seems generally to assume that "prices" (revenues from sales) is supposed to cover the costs of R&D already incurred for that particular product, which is a misunderstanding or at least an over-simplification, as these costs are "sunk". Instead, revenues from sales of marketed products are re-invested into research of future products and innovations.

Line 2791ff: the recommendation to "enforce transparency […] around the price of medicines" may be counterproductive in the context of External Reference Pricing and Parallel Trade as it may lead to access delays and shortages.[19] In addition, price transparency would undermine flexible approaches to provide access, and several Member States are against disclosing net prices.[20] It is also not supported by economists.[21] Current discussions about pharmaceutical pricing however show that there is an information gap between industry and policy makers and other stakeholders.

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[17] See: http://www.biocentury.com/antibioticsncepipeline.htm (accessed: 20/10/2015)

[18] http://www.pewtrusts.org/en/research-and-analysis/issue-briefs/2014/03/12/tracking-the-pipeline-of-antibiotics-in-development (accessed: 20/10/2015)

[19] See e.g. Ridley DB (2005), Price Differentiation and Transparency in the Global Pharmaceutical Market Place; Pharmacoeconomics 23 (7): 651-658: "More significantly, the WHO efforts to increase transparency are likely to


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A broader discussion about the business model of the industry would be welcomed but may go beyond this opinion. The proposal to use mandatory licences should not be a solution to address pricing issues at the detriment of innovation. Such a policy would not only be at odds with the objectives of promoting investments in health and in R&D but would also greatly jeopardise the EU position as a leader in life sciences and innovation.

Line 2796 and 2829: no background has been given in the report to this policy recommendation, so it is difficult to understand the reasoning behind this. Horizon 2020 is, like national research programs, mainly funding basic, non-competitive research in a broad range of sectors, so it's difficult to understand how this would be linked to specific products. The concept "socially responsible licensing" is also not clear.

Line 2799: as stated above, EFPIA supports evidence-based decision-making, both at launch and over time. Evidence produced through HTA will however not replace decisions which have to be taken by decision-makers. Furthermore, EFPIA caution that HTA is automatically linked to the concept of cost-effectiveness analysis. Budget impact is much more relevant for decision-makers then artificial concepts of cost-effectiveness. Sustainable healthcare systems should discuss investments in innovation in the context of a manageable budget impact of pharmaceuticals.

Line 2802: while **joint procurement** may have benefits in some situations and for certain medicines, the recommendation in this opinion requires further explanation. **It is crucial to ensure that joint procurement arrangements do not jeopardize sustainability and supply of innovative medicines.** Joint procurement should foster effective competition and not distort it by de facto limiting the number of suppliers. Avoiding excessive concentration of purchasing power will therefore be important to ensure the ability to respond to the Member States needs. In markets functioning by public procurement, a supplier who loses a public bid thereby loses all or nearly all access to the market for the duration of the tender, which could go up to several years. The decrease in demand resulting from this exclusion may push a supplier below the level of production necessary to sustain the high fixed costs of continued production. The aggregation of demand could potentially magnify these elements and drive some suppliers completely out of the market. Thus the concentration of demand could increase the risks inherent in the innovative medicines business and endanger the sector's sustainability.

Line 2817ff: though the patent system often gets criticised for the wrong reasons, **EFPIA emphasis that pricing mechanisms and the patent system are distinct and separate entities**. Prices are set up according to specific criteria and in negotiation with the relevant authorities and buyers at the Member States' level. The patent system has no bearing on how prices are fixed nor at which level they are fixed. As a consequence, and as noted previously, EFPIA believes it is inappropriate to blame the patent

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lead to less price differentiation and less access to innovative pharmaceuticals. An important reason why manufacturers are reluctant to charge lower prices in lower-income countries is that they fear that such low prices will undermine the prices they charge to higher-income consumers."

[20] See e.g. APM (15/10/2015), Head of Germany's GBA welcomes insurer's proposal on 'secret' discounts

[21] See e.g. Danzon PM, Towse A (2003), Differential Pricing for Pharmaceuticals: Reconciling Access, R&D and Patents; International Journal of Health Care Finance and Economics, 3, 183–205


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system or refer to "patent misuse" to criticise prices or the lack of detailed knowledge of costs and prices, when none of these are objectives of or consequences of the patent system.

Line 2822: the European Parliament has initiated several reports on medicines, access and affordability. Nevertheless, European Parliament is a political body and may lack the expertise to assess prices (e.g. compared to a pricing and reimbursement agency). In addition, pricing and reimbursement of medicines is a national competence, this also to ensure that national requirements are reflected. A **European Parliament body would interfere with national competencies and may delay access to new medicines**.

Line 2826ff: the SPC Regulation Preamble (Recitals 3-5) recognized that the long and costly research needed as well as the regulated nature of our industry resulted in a lack of protection, which "penalizes pharmaceutical research", concluding that "medicinal products […] will not continue to be developed in the Community and in Europe unless they are covered by favorable rules that provide for sufficient protection to encourage such research". This was the purpose of the SPC Regulation, which took into account "all the interests at stake, including those of public health" (Recital 10) to come up with the 15-year maximum effective protection period, compared to the internationally agreed 20-year patent term. It is believed that the SPC Regulation struck a fine balance between these interests.

Line 2832ff: EFPIA believes the opinion fails to recognise the necessary balance between a number of different objectives. It is not in dispute that prices and access may be debated, but **the question is what should the policy responses be and whether measures to weaken the existing incentive system and risk innovation are appropriate**, given that no suitable and credible alternative has been proposed e.g. no credible system to de-link R&D has so far emerged or proved capable of supporting the costs and risks associated with the development of clinical trial process and to bring new medicines to patients.

Section 7 People can use services when they need them

Lines 3088ff: we agree with the opinions view about the importance of health literacy. Health, health literacy and the socio-economic status ("social gradient") are closely linked and require a broader policy approach beyond health policy. **Access to healthcare depends on the navigation skills of the users, on the one hand, it also depends on the "readability" of the health system**.[22] Improving health literacy is in particular relevant in the context of managing chronic diseases.

Section Access for Roma, undocumented migrants and people with mental health problems

Lines 3841ff: we agree with the opinion that specific strategies are needed to improve the access to appropriate health services for people with mental health disorders. In addition to the suggestion that improving prevention and early detection, it would be relevant to also reflect comments and suggestions from the OECD 2014 Report "Making Mental Health Count: The Social and Economic Costs of Neglecting Mental Health Care". Some suggestions made include:

1) Matching spending on mental health against the need (e.g. mental health is 23% of England's total burden of disease, but receives 13% of the National Health Service health expenditure)
2) Improving access to psychological therapies to help close the large treatment gap.

[22] See e.g. Kickbusch I and Maag D (2008), Health Literacy. In: Kris Heggenhougen and Stella Quah, editors International Encyclopedia of Public Health, Vol 3. San Diego: Academic Press; pp. 204-211.


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3) Including employment as an important outcome in mental health
4) Increasing the scope for GPs to be involved in the ongoing care of people with severe mental illness.
5) Align provider incentives with desired mental health outcomes
6) Better data collection to track quality of mental health treatment

Section "Ensuring equitable access: EU and Member State responsibilities and responses"

Lines 4091ff.: "It [i.e. improving affordability] also has particular resonance when it comes to access to medicines, especially (but not only) new and innovative medicines, which are increasingly priced beyond the reach of many countries, including countries in the European Union." – As mentioned above this statement seems to indicate that medicines would be paid directly by patients; in nearly all European countries, medicines – as long as they have undergone P&R assessments – are covered by public health insurance, in particular so-called "high-price medicines". In addition, **the statement does not take into account that industry and payers/ governments in many Member States have found flexible access schemes which ensure affordable access to new medicines**.

Lines 4192ff: it would be relevant to **further specify the broad lines of what such a national public health strategy should entail in response to which gaps**. This is considered of fundamental importance as most EU countries have no vaccination plan to identify their need for new / improved vaccines within the medium (3 to 5 years), and long terms (10 years). The unmet need is not specified and a priority list is needed to facilitate rational vaccine development and prioritisation. Moreover there is a lack of valid epidemiological data in most countries, as well as comprehensive data on vaccination coverage as to inform and tailor appropriate public health strategies.


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