

Core Principles from the Patients’ Perspective on the Pricing and Reimbursement of Innovative Medicines

April 2016

This document has been prepared by the EPF board and supported by our membership¹ as a constructive contribution to the current EU level debates on pricing and reimbursement. A further consensus document will be published in June 2016, that will explore further some of the elements described in this preliminary document.

The objective of this document is to:

- Outline the key concerns of the patient community regarding current pricing and reimbursement models for medicines that contribute to inequities in access
- Provide a patient perspective on the current debate on the value of innovation
- Highlight key principles that the pharmaceutical industry, political decision-makers in Member States and the EU Institutions should apply in order to ensure equitable access to treatment based on need, not means, while supporting the long-term sustainability of our health systems.

Introduction / background

The European Patients’ Forum’s vision is that all patients with chronic and/or lifelong conditions in the EU have access to high quality, patient-centred equitable health and social care. Timely access to accurate diagnosis and appropriate treatment not only improves

¹ Due to the political situation and the upcoming Referendum in the United Kingdom, our member organisation AMRC is not in a position to endorse this statement, as it cannot endorse any EU initiative at this juncture.

patient health and well-being but can also offset significant costs to the health and social systems as a result of avoidable exacerbation of the health condition.

Access to healthcare, including to medicines, is recognised as a fundamental right. Inequity of access is a main factor behind health inequalities^{1,2} and the European Parliament has recognised patients with chronic diseases as a group whose needs should be taken into special consideration when addressing health inequalities.³

Particularly given the ongoing impact of the financial crisis, there is an urgent need to identify effective and concrete actions at EU level to close the current gap on health inequalities, so that all patients across Europe have access to high-quality, affordable treatments. The disparities in access predate the financial crisis in Europe, but they have been made worse against a background of austerity measures and falling healthcare spending in many Member States since 2009.⁴ Patient groups perceive austerity policies as characterised by short-term measures – cuts in healthcare budgets and insurance coverage, increased patient fees and co-payments, and cuts in social protection measures.

The wide divergences across the Union in patients' access to treatment are in EPF's view unjustifiable. They are contrary to the principles set out in the Charter of Fundamental Rights, the EU Treaty's commitment to the principle of well-being,⁵ and to the fundamental European values of equity, solidarity and good quality in healthcare.⁶

For patients with chronic diseases, medicines form an important, sometimes crucial, aspect of treatment. Innovative medicines hold the promise of cure or at least significant improvement to health or quality of life.

The "pipeline" of the pharmaceutical industry has changed. On the one hand, as many so-called blockbuster medicines have or are soon reaching the end of their exclusivity period, there is a space for more generic medicines to enter the markets. This is good news for patients as generics entry lowers prices for the healthcare system and increases the range of available therapeutic options.⁷

On the other hand, scientific knowledge is advancing very fast. New, potentially groundbreaking discoveries are being made, including in personalised medicine and genomics.⁸ Industry is increasingly focusing on developing differentiated and specialised medicines targeted at smaller patient groups. This is also good news, as it holds the promise of more effective and personalised solutions to patients' specific needs. Yet, these are often extremely expensive. Patients will only reap the benefits of the new therapies if, once they are on the market, they are accessible and affordable to all who need them.

Access to medicines can be framed in similar terms as access to healthcare generally, on the principle of "equitable access based on needs not means":

- *Availability*: the medicine needs to be available in the market;
- *Affordability*: patients should not suffer financial hardship as a result of seeking treatment;
- *Adequacy*: the medicine should be safe, of high quality and effective;
- *Appropriateness*: the medicine needs to be appropriate for patients' needs;
- *Accessibility*: the medicine should be distributed through reachable channels, without geographical or time barriers.⁹

DEVELOPMENTS AT EUROPEAN LEVEL

The updated report *Priority Medicines for Europe and the World* (WHO, 2013), the reports of the Belgian EU presidency on *Innovation and Solidarity* (2010), and the Council Conclusions on *Innovation for the benefit of patients* (2014) have raised concerns about access and questioned the nature of innovation: what does “innovative” mean; what should be considered “valuable” innovation; and how should valuable innovation be adequately incentivised and rewarded?

This question is becoming increasingly urgent, as unacceptable disparities in patients' access to medicines persist, whilst healthcare systems have been struggling to accommodate the cost of some new medicines.

At the same time, many are concerned that the decisions taken on pricing and reimbursement are hampered by lack of transparency on the real costs of medicines. The Council conclusions on *Innovation for the benefit of patients* (December 2014) called for greater collaboration on HTA as well as more “effective sharing of information on prices of and expenditure on medicinal products, including innovative medicinal products”.

Practices such as “ever-greening” of patent protection and deals to prevent or delay the entry of generic products onto the market, and “price-gouging” – whereby certain companies acquired old, neglected drugs and turned them into costly ‘new’ drugs¹⁰ – have drawn attention to failures and gaps in the current system and reinforced calls on the pharmaceutical industry to “walk the talk” as a responsible health stakeholder.

Member States of the EU have committed to the UN Sustainable Development Goals, which comprise achieving universal health coverage, including financial risk protection, access to quality essential health-care services and access to safe, effective, quality and affordable essential medicines and vaccines for all by 2030.

The European Commission recently set up an expert group on *Safe and Timely Access to Medicines for Patients* (STAMP)¹¹, where experts from Member States exchange information about the experience of Member States, examine national initiatives and identify ways to use

more effectively the existing EU regulatory tools with the aim to improve patients' access to medicine. STAMP has been looking at key issues such as adaptive pathways in 2015.

Although pricing and reimbursement of medicines remains a national competence, we believe there is value in closer European collaboration. More action is urgently needed at European level to drive change. Patients constitute the most important stakeholder group in this – both from a moral perspective, because pricing and reimbursement policies directly impact their lives and well-being, but also from a practical perspective, because a meaningful definition of “value” and “added therapeutic value” is only possible with the involvement of patients.

Current pricing models do not work

Many now believe that new medicines are too expensive and threaten equity of access but also the long-term sustainability of European health and social systems. Public healthcare systems are confronted by conflicting aims – of providing equitable access to innovative medicines, whilst also preventing escalation of the costs. Unfortunately, too often they try to balance this by passing more of the cost of the medicines on to patients – through restricting reimbursement or increasing co-payments – strategies which are not only unacceptable from an equity perspective but also counterproductive, as they exacerbate societal inequalities. Another strategy used is rationing of patients' access, whereby only some of the patients who could benefit from the medicine have access to it.

In an effort to try to drive down the prices of medicines, member states practise **external reference pricing (ERP)**.¹² Whilst this may generate savings at least in the short term, it has significant disadvantages for patient access, particularly in poorer member states: companies adapt their market strategies to launch products in high-paying countries first, creating delays in patients' access in other markets. Sometimes they decide not to launch a product at all if it is not deemed commercially worthwhile. ERP may also lead to price convergence, which can disadvantage low-income countries who will pay a higher price than if they did not use reference pricing.¹³

Differential pricing has been put forward as an alternative strategy, both by the pharmaceutical industry and some other stakeholders.¹⁴ Differential pricing is a strategy whereby price is set according to different economic situations between countries. Until now, it has been discussed mainly in terms of “price discrimination” – a type of pricing strategy adopted by companies to optimise market access. However, differential pricing as a *political strategy* driven by collaboration between member states with the aim of improving equity of access, has been less explored.

Recently, attention has shifted towards the concept of **value-based pricing**. This is based on the idea that prices of medicines should be linked to the health benefit they bring to patients and to society. It is framed by broader discussions on the need to measure health outcomes generally motivated by the desire to improve the quality of care and public health as well as foster greater transparency and accountability of the health systems. It is also believed that value-based pricing may help balance short-term cost effectiveness with long-term incentives for industry to develop added-value products. EPF is contributing to reflections on health outcomes, together with other stakeholders.¹⁵

According to a 2013 report by the OECD, many countries already apply some type of “value-based” policies in pricing and reimbursement, but the way value and cost-effectiveness are determined and the different factors applied in assessment is very different in countries around the world. There is also no commonly accepted definition of value.¹⁶

EPF believes that future approaches towards pricing and reimbursement will need to be linked to the (added-) value of a medicine, and to involve some degree of differentiation. We believe the constructive discussion at European level should continue, aiming for a political consensus on the principles, scope (for example, specific medicines for high unmet needs), and a strategy for tackling practical issues. This discussion requires political will and leadership, and a commitment to solidarity and ethics from the member states and industry, and it must take place with the involvement of patients and (public) payers.

One major barrier for differential pricing is the existence of **parallel trade**. Parallel trade is based on the principle of free movement of goods within the internal market, but it is a factor in medicine supply shortages, reduced access and safety concerns. EPF believes that Member States should prioritise the interests of patients’ and public health over the internal market, and take action to limit parallel trade where necessary. In addition, member states would have to limit external referencing for those medicines where a differential strategy would be adopted.

Transparency of prices – or of pricing decisions?

More **transparency on medicines prices** has been mooted as a solution for making medicines pricing more realistic and more equitable. Essentially, the argument is that external reference pricing would work if member states could know the actual prices paid by other member states. At EU level, the EURIPID project has set up a price database for competent authorities, but until now it only includes list prices, not actual prices. These are achieved as a result of negotiations with companies, which are confidential.

Some stakeholders argue that there should be no secret deals between payers and companies, and the real prices should be shared so member states could drive down prices. The pharmaceutical industry, in turn, argues that full transparency would be counter-productive for access.¹⁷

In principle, we believe in progress towards maximum transparency for reasons of openness and accountability of the system towards patients and citizens. Value-based pricing and implementation of performance-linked risk-sharing agreements also imply the need for more transparency. However, we believe more knowledge is needed on the possible unintentional or undesirable impacts of transparency of actual negotiated prices on patient access and how these might be managed.

Availability of information on the *processes* and *criteria* of decision-making on pricing and reimbursement also varies, and some national authorities are not adhering to the *timelines* of 180 days for decision-making under the EU Transparency Directive.¹⁸

From a patient's perspective, the divergent decisions reached by HTA bodies of different member states, and sometimes within the same member state¹⁹, on the same medicines is confusing and leaves patients in an unequal situation. Patients are also not always aware of what medicines are being assessed, what criteria are used, and who is involved. They also do not know how to engage in the process. EPF believes patients can make a meaningful contribution to this process and their involvement should be integrated and supported.

Assessing the true value of innovation

In EPF's view the word innovation carries with it normative connotations. We understand an innovative medicine to be a new medicine that brings added value for patients.²⁰ Not everything that is new can be considered innovative in this positive meaning of the word, even though the medicine's molecular structure or mechanism of action could be new. What matters is whether the medicine makes a tangible (positive) difference for patients.

The concept of *added therapeutic value (ATV)*, at the heart of the current discussions around medicines pricing, refers to a therapeutic advantage offered by a new medicine compared to existing ones, but there is no universally agreed definition of the concept. A recent European Parliament study defined it as "the incremental 'therapeutic value' brought by a new drug or intervention compared with the best available treatment options already on the market. The therapeutic value can be defined in terms of patient-relevant endpoints and relevant levels of effectiveness, efficacy, and safety."²¹

The added therapeutic value of innovative medicines is debated; some sources claim that most new medicines do not add anything significant to the existing treatment options, and some may even do more harm.²² We believe it is fundamentally unethical to spend limited public funds on therapies that do not have demonstrable added value. However, it is difficult to comment conclusively on assessments on specific medicines, as long as it is not clear how they incorporated the patient’s perspective of the balance between benefits and harms. It is well known that patients can have a very different perspective of therapeutic benefit, different priorities (both in terms of quality-of-life factors and prioritised clinical outcomes), and different levels of acceptance of potential risks, compared to medical professionals.²³

MORE COLLABORATION ON HEALTH TECHNOLOGY ASSESSMENT – WITH PATIENTS

Health Technology Assessment (HTA) is used to assess the relative effectiveness of a new medicine compared to existing ones, supporting decisions on pricing and reimbursement that are meant to be fair for patients and for society. In its broad form, HTA is meant to be “a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value.”²⁴

However, HTA as currently practised tends to be a much less broad exercise, aiming to reach a decision quickly and prioritising cost-effectiveness *over other factors*, such as the value itself, ethical and societal benefits. Moreover, different member states weigh these factors differently.

We do believe that closer **collaboration on HTAs and relevant methodologies** will help address the current fragmented situation. European collaboration on *so-called “rapid REA”* can help improve patient access if it focuses solely on scientific/clinical data where there are no significant member state specific elements; if the process starts early enough so that the joint report is available at the same time as the marketing authorisation; and if the joint assessment is fully implemented by member states, rather than adding an extra element into the national process. Patient involvement in rapid REA also needs to be mapped out as part of the health technology assessment framework.

The importance of **incorporating the patient perspective** in HTA is increasingly recognised,²⁵ and the integration of quality of life factors in HTA is considered necessary in order to arrive at an accurate assessment of a medicine’s added value. But in practice, patient involvement in HTA is still very limited, and so far there is no agreement on the best method of involving patients.²⁶

In our view, the value of an innovative medicine for patients needs to be always at the heart of HTA. The patient experience may be difficult to capture fully in formal (quantitative) measures, and therefore qualitative evidence also needs to be integrated. Taking as starting points initiatives such as the HTAi Patient and Citizens' Sub-Group and existing best practices in HTA agencies across the world, appropriate methodologies and structures can be developed.

Towards flexible life-cycle approaches

Scientific advances in the area of genomics and personalised medicine are likely to have a major impact on medicines development, authorisation, pricing and reimbursement in the longer term as science moves towards more targeted populations, precision /personalised therapies, and predictive medicine.

The concept of **adaptive pathways** approaches therapeutic R&D, licensing and evaluation as one continuum over the 'life-cycle' of the medicine: early dialogue and scientific advice during the R&D process; possible conditional authorisation/adaptive licensing; flexible pricing and reimbursement models; continuous monitoring of health outcomes; and regular re-assessments. This implies the need for more collaboration between the pharmaceutical, med-tech and IT industries, regulators, HTA, payers, medical professionals and patients. ADAPT-SMART, a project funded under the EU Innovative Medicines Initiative (IMI-JU) is currently exploring the scientific, practical, ethical and legal aspects of adaptive pathways.²⁷

EARLY DIALOGUE

Collaboration between all of the stakeholders at EU level is needed from the earliest stages of medicines research and development. This will help define anticipated benefits and value, outcomes for patients, and more reliable basis for value-based pricing. It will make the process of R&D more predictable for the industry and could enable member states and patients to have more of a steering role on the R&D priorities of companies.

CONDITIONAL AUTHORISATION

EPF believes adaptive pathways have potential in future to promote faster access to beneficial treatments for the right patient groups. Adaptive pathways aim to balance earlier patient access with an acceptable benefit-risk balance. A medicine would be authorised in a small patient group where it has clearly demonstrated efficacy and safety, *on the condition* that further evidence is systematically collected and regularly evaluated. The authorisation may be extended at a later stage, if warranted by the evidence. In order to implement an adaptive

approach properly, a careful balancing exercise is needed on the benefits and risks involved, defining levels of acceptable uncertainty, criteria for using the approach, patient protection, and possible exit strategies. In EPF's view, this approach is currently appropriate for patients with very serious illness where no other treatments are available. To be successful, the adaptive approach needs to be centred around the patient perspective on benefits and risks.

CONTINUOUS MONITORING AND REAL-WORLD EVIDENCE

Real-world evidence is key to the success of adaptive pathways: robust systems must be in place for post-market data collection (on ADRs, changes to the benefit-risk balance, health outcomes). Real-world evidence is also key to more *re-purposing of existing products*, thus potentially bringing new treatments to area of unmet need. This is likely to become more important with personalised medicines, as diagnostic technologies will enable subgroups of patients who will benefit to be identified more accurately. The necessary infrastructure must be put in place for systematic collection of real-world evidence, particularly through well-designed, interoperable registries, to capture treatment outcomes both successful and unsuccessful, and including off-label use. The information collected must be quickly available for appropriate action to be taken by regulators, industry, medical professionals and patients.

MANAGING UNCERTAINTIES

To manage the risks associated with uncertainties regarding the value of new medicines compared to the high prices, Member states are increasingly using a variety of tools that collectively referred to as *managed entry agreements* (MEAs). These can range from instruments with a rather narrow financial focus such as rebates and discounts linked to price-volume agreements and capping schemes, to more outcomes-focused approaches such as conditional reimbursement subject to reassessment, and coverage with evidence development, where the company is obliged to provide additional data on real-life performance of medicine. They all have the common aim of facilitating access to new medicines in a context of uncertainty and high prices.²⁸

From the patient perspective, purely financial measures are problematic as they are essentially sticky-patch solutions, reflecting failures of the system. They are also focused on the short-term, and their benefit appear dependent on the secrecy of negotiations. On the other hand, MEAs aimed at collecting evidence of treatment outcomes over a longer time and linking pricing and reimbursement to added value are more interesting. They are aimed at managing uncertainties and at ensuring that the right patients benefit from the treatments. We believe such approaches should be studied further. The right infrastructure and frameworks need to be in place to ensure the systematic collection of necessary data. Pricing

and reimbursement decisions would need to be regularly revisited as new evidence accumulates, and would need to be flexible.

Joint price negotiations – a way forward

In May 2015 EPF and EURORDIS published a joint letter calling on the EU’s pricing and reimbursement authorities to support the establishment of a “table for price negotiation” with a group of Member States, and scaling-up of the pilots on early dialogue between payers and developers of medicinal products. We believe this would lead to better collaboration between industry and payers and, ultimately, to better access to medicines and improved health outcomes. It could be established first by a core group of “willing Member States” and progressively integrate more countries.²⁹ There is already interest among some member states in collaboration; the Netherlands, Belgium and Luxembourg recently agreed to start joint negotiations of prices for some orphan drugs, aiming for enhanced exchange of information and joint work on the assessment of medicines.³⁰ Similar discussions have started between Bulgaria and Romania.³¹

This approach is only possible if all parties accept that pricing discussions will be based on a value assessment – especially for products in areas with small populations and high uncertainties – and will be linked to post-market evidence generation. Prices would have to be flexible over time – it would need to be possible to adjust them both up and down.

Naturally several factors will play a part in the value assessment, including value for patients, value for money (cost-effectiveness) and budget impact. A company should not be able to dictate its price completely freely, even for medicines that do represent valuable innovation, if this makes the innovative medicine unaffordable and inaccessible.

The bigger picture

Research and development in pharmaceuticals should be geared towards unmet needs of patients and of public health, including those in the updated WHO 2014 Report on Priority Medicines. We believe more open debate is needed around investment in health, valuable innovation, as well as societal values and preferences. Patients and the public play different and complementary roles in these debates: whilst a broad discussion is needed around societal values, priorities and public health needs, when it comes to identifying unmet needs at individual patient or disease-level, it is vital to involve patients and their organisations.

It is natural for commercial companies, whose priorities are driven primarily by shareholder value, to be focusing on what is commercially attractive rather than public health needs per se. However, the commercially-driven model is not providing needed innovation for many critical health needs, in particular poverty-related and neglected tropical diseases as well as antibiotics.³² A “correction” of the research priorities is needed, for example through more publicly funded research (including basic research, comparative research on existing treatments and treatment combinations, and repurposing). This investment should be fully reflected in the price of the final medicine.

EPF believes there is a role for developing genuine Public Private Partnerships in addressing unmet needs that can foster innovation.³³ More broadly, EPF welcomes emerging initiatives looking at alternative funding models for pharmaceutical R&D, such as patent pools and other “de-linking” initiatives, which may be particularly relevant when it comes to addressing global health needs and health equity.³⁴

In addition, other issues that need to be addressed to improve cost-effectiveness of care and outcomes for patients and society: reducing waste in health systems, including the use of inappropriate therapies or services; patient adherence;³⁵ corruption, which is an important access barrier in some member states; and unethical practices by some companies, which should be monitored and strictly sanctioned.

Core principles on pricing and reimbursement

1. Medicines are not a consumer good like any other; patients are not merely consumers. Medicines are an essential element of health policy. Health is a fundamental right as well as a critical investment in the well-being, economic development and cohesiveness of society.
2. There is an urgent need to develop a coherent a framework for “fair access” to innovative medicines across the EU that maximises societal benefit and patient access whilst avoiding unacceptable cost impact on healthcare budgets. Such a framework should encompass multiple elements in flexible combination to accommodate the differences in EU health systems.
3. A common understanding is needed on the concepts of “innovation”, “value” and “added therapeutic value”. Patients’ views should be central to this understanding, including patients’ perceptions of quality of life and patient-relevant clinical endpoints.
4. Common principles should be adopted to encouraging and rewarding innovation in order to encourage investment in R&D. Societal debate is needed on what constitutes a “fair” return on investment and reward for added value. Such a debate implies the need for more transparency about the real costs of medicines’ research and development.
5. Pharmaceutical companies should price new medicines responsibly to ensure that they are accessible and affordable to all who need them. Medicine prices cannot simply be based on what the market can bear: pricing should also consider other factors, e.g. a country’s relative capacity to pay; budget impact; and the extent of public funding that contributed to the development of a medicine.
6. Frameworks, structures and methodologies should be developed for meaningfully incorporating patient evidence into Health Technology Assessments and REAs, including joint multi-country assessments.
7. Patients should be considered a key stakeholder group in medicines pricing and reimbursement; they should be involved in a structured way through their representative organisations in pricing and reimbursement at national level.
8. Pricing and reimbursement authorities should be transparent towards patients about how they make their decisions, what criteria they use, and who is involved in the process. Information explaining decisions should be available in an easily accessible and understandable format that addresses the specific questions of patients.
9. Patients’ needs go beyond medicines and include other therapeutic options, community support, and so on. Innovation should be encouraged in this wider sense, encompassing better ways of structuring and delivering integrated health and social care; more efficiency and effectiveness; social innovation; and the development and effective use of new user-driven technologies.

Notes and references

¹ Communication from the Commission to the European Parliament, the Council, the European Economic and Social

Committee and the Committee of the Regions. "Solidarity in health: Reducing health inequalities in the EU". Brussels, 20.10.2009. COM(2009) 567 final.

² See EPF's position statements on health inequalities, available at our website: www.eu-patient.eu/Initatives-Policy/Policy/Health-inequalities/

³ European Parliament resolution of 8 March 2011 on reducing health inequalities in the EU (2010/2089(INI))

⁴ OECD (2013) Health at a Glance 2013: OECD Indicators, OECD Publishing.

http://dx.doi.org/10.1787/health_glance-2013-en; Eurofound (2014), Access to healthcare in times of crisis, Publications Office of the European Union,

Luxembourg. www.eurofound.europa.eu/sites/default/files/ef_publication/field_ef_document/ef1442en.pdf

⁵ Article 3, Treaty on European Union

⁶ Council Conclusions on Common values and principles in European Union Health Systems (2006/C 146/01)

⁷ See e.g., IMS Institute (2015) "The Role of Generic Medicines in Sustaining Healthcare Systems: A European Perspective."

⁸ "Shaping Europe's Vision for Personalised Medicine. Strategic Research and Innovation Agenda (SRIA)". Coordination & Support Action (CSA) PerMed, June 2015. Available at www.permed2020.eu

⁹ Adapted from EPF position paper "Defining and Measuring Access to Healthcare: the Patients' Perspective," March 2016. Available at www.eu-patient.eu/whatwedo/Policy/access-to-healthcare/

¹⁰ "Drug Goes From \$13.50 a Tablet to \$750, Overnight" New York Times, 20 September 2015; "Big Price Increase for Tuberculosis Drug Is Rescinded", New York Times, 21 September 2015.

¹¹ http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm

¹² For definitions and descriptions of different pricing models, please see "Study on enhanced cross-country coordination in the area of pharmaceutical product pricing", final report under contract number 2014 73 03 for the implementation of Framework Contract № EAHC/2013/Health/01 'Health economic reports – analysis and forecasting' (Lot 2), available at

http://ec.europa.eu/health/systems_performance_assessment/docs/pharmaproductpricing_frep_en.pdf

¹³ EC study on pharmaceutical pricing. See reference 11.

¹⁴ "Improving Patient Access to Innovative Medicines the Framework in Which Differentiated Pricing May Offer a Solution", EFPIA 2014 position paper. Available at www.efpia.eu/uploads/Modules/Documents/pac-280214-ai6-a1-differentiated-pricing-position-paper-final.pdf

¹⁵ "Enhancing Value in European Health Systems. The Role of Outcomes Measurement". Multi-stakeholder consensus document (2016). Available at www.eu-patient.eu/globalassets/policy/patientssafety/value-of-health-consensus-document.pdf

¹⁶ Paris V and Belloni A (2013) "Value in pharmaceutical pricing", OECD health working papers, No. 63, OECD Publishing, pp. 11-14.

¹⁷ EFPIA position paper, principle 1. See reference 13.

¹⁸ EPF statement on the European Commission's proposal for a review of the Transparency Directive, November 2012.



¹⁹ As an extreme example, in March 2016 in the UK, the Scottish HTA body rejected a treatment for advanced skin cancer because of uncertainty around the long-term benefits, considering it not a good use of NHS resources. Whereas in January of 2016, NICE (the HTA body in England) had approved the same treatment.

²⁰ EPF's comments on the European Medicines Agency Strategic Framework 2020. Available at www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000292.jsp&mid=WC0b01ac05800293a4

²¹ "Towards a Harmonised EU Assessment of The Added Therapeutic Value of Medicines". Study for the ENVI committee. Directorate General for Internal Policies, Policy Department A: Economic and Scientific Policy, 2015, pp. 14-15. Available at [www.europarl.europa.eu/RegData/etudes/STUD/2015/542219/IPOL_STU\(2015\)542219_EN.pdf](http://www.europarl.europa.eu/RegData/etudes/STUD/2015/542219/IPOL_STU(2015)542219_EN.pdf)

²² "Added Therapeutic Value: European citizens should get their money's worth". Joint position paper by WEMOS, EPHA, SOMA and ISDB. 2014. Available at www.wemos.nl/files/Documenten%20Informatief/Bestanden%20voor%20'Medicijnen'/Position%20paper%20ATV%20Wemos%20SOMO%20EPHA%20ISDS.pdf

²³ See for example: "New Medicines for Serious Conditions: How patients would weigh the risks and benefits," Report of Findings from Phase 1 and 2. Genetic Alliance UK in partnership with the Welsh Institute for Health and Social Care, University of South Wales. April 2014. Available at www.geneticalliance.org.uk/docs/risks-and-benefits-2-report.pdf

²⁴ EUNETHTA definition, available at www.eunetha.eu/about-us/faq#t287n73

²⁵ EU HTA Network: "Strategy for EU Cooperation on Health Technology Assessment", 2014, p. 8. Available at http://ec.europa.eu/health/technology_assessment/docs/2014_strategy_eucooperation_hta_en.pdf

²⁶ EPF survey: "Patient Involvement in Health Technology Assessment in Europe," 2013. Available at www.eu-patient.eu/whatwedo/Initiatives/

²⁷ <http://adaptsmart.eu/>

²⁸ For an overview of MEAs, see Ferrario, A and Kanavos, P (2013) "Managed entry agreements for pharmaceuticals: the European experience." EMiNet, Brussels, Belgium. Available at http://eprints.lse.ac.uk/50513/1/_Libfile_repository_Content_Ferrario%2C%20A_Ferrario_Managed_%20entry_%20agreements_2013_Ferrario_Managed_%20entry_%20agreements_2013.pdf

²⁹ www.eu-patient.eu/globalassets/alliance-building/eurordis-epf-letter_call-on-payers_may2015.pdf

³⁰ www.deblock.belgium.be/fr/grand-duchy-luxemburg-joins-belgium-netherlands-initiative-orphan-drugs

³¹ <http://www.eiu.com/industry/article/723225456/teaming-up-for-pharma-bargains/2015-06-04>

³² Kaplan W et al., (2013) Priority Medicines for Europe and the World. 2013 update. World Health Organisation, Chapter 6.

³³ Ibid., Chapter 8.1.

³⁴ For example the United Nations High – Level Panel on Access to Medicines. www.unsgaccessmeds.org

³⁵ For a patient perspective on adherence, see "Adherence and Concordance. EPF position paper" (2015) available at www.eu-patient.eu/globalassets/policy/adherence-compliance-concordance/adherence-paper-final-rev_external.pdf

