

Core Principles from the Patients' Perspective on the Value and Pricing of Innovative Medicines

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This document has been prepared by the EPF board and supported by our membership as a constructive contribution to the current EU level debates on the value and pricing of innovation. It builds on a preliminary statement circulated in advance of the informal Health Council in mid-April.

The objective of this document is to:

- Outline the key concerns of the patient community regarding inequities in access related to current pricing models
- Provide a patient perspective on the current debates on the value of innovation
- Highlight key principles that political decision-makers in Member States, the EU Institutions and the pharmaceutical industry 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

The European Patients' Forum's vision is that all patients with chronic and/or lifelong health conditions in the EU have access to high quality, patient-centred equitable health and social care. Access to healthcare, including to medicines, is recognised as a fundamental right and promoted as a common EU policy. Inequity in 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

Timely access to accurate diagnosis and appropriate treatment not only improves patient health and well-being but can also offset significant costs to the health and social systems as a result of avoidable exacerbation of health conditions. For patients with chronic diseases, medicines form an important, sometimes crucial, aspect of treatment. New and better medicines hold the promise of significant improvements to health or quality of life or even a new lease on life in the form of a cure.

Access to medicines can be framed in similar terms as access to healthcare generally, following EPF's 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 and healthcare systems should not suffer financial hardship as a result of seeking to provide treatment for their citizens;
- *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.⁴

Accessibility is becoming increasingly urgent in health and social policies. Unacceptable disparities in patients' access to medicines persist, whilst healthcare systems are increasingly struggling to accommodate the cost of some new medicines. Existing disparities have been made worse against a background of austerity measures and falling healthcare spending in many Member States since 2009.⁵ Patient groups perceive such austerity policies as being short-term budgetary gains – cuts in healthcare and insurance coverage, increased patient fees and co-payments, and withdrawal of social support and protection.

Contributing to this development are changes in the industrial research and production process of new medicines. On the one hand, as many so-called “blockbuster medicines ” have reached or are soon reaching the end of their period, there is a growing opportunity for new generic medications to enter the markets. This can be seen as good news for patients as generics entry can considerably lower the prices paid by healthcare systems by introducing competition.⁶ On the other hand, scientific knowledge is advancing fast. New, potentially ground-breaking, discoveries are being made, including cures from chronic infection and in personalised medicine and genomics.⁷ An increasing number of research projects and companies are 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 new treatments 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.

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’ drugs⁸ – 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. Prices of medicines cannot simply be based on strategies to maximise profit without ensuring access to all who need them.

Member States of the EU have all 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. Inequalities in access are contrary to these goals, to the Charter of Fundamental Rights, to the EU Treaty's commitment to the principle of well-being,⁹ and to the fundamental European values of equity, solidarity and good quality in healthcare.¹⁰

EPF therefore believes there is an urgent need to react to these developments, to identify effective and concrete solutions at EU level and to ensure that all patients across Europe have access to high-quality, affordable treatments.

Current pricing models do not support equitable access

Many now believe that new medicines are too expensive and threaten not only equity of access but also the long-term sustainability of European health and social systems. Healthcare systems are confronted by conflicting aims – providing equitable access to innovative medicines, whilst preventing an unacceptable escalation of 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 worsen health outcomes and exacerbate societal inequalities. Similarly rationing, i.e. limiting access to a defined number of patients for entirely economic reasons, undermines the principles of solidarity and equity. It existentially threatens the interests of the patients denied access despite medical needs.

In an effort to try to drive down the prices of medicines, Member States practise **external reference pricing (ERP)**.^{i,11} Whilst this may generate savings and room for shifting costs 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 countries. Sometimes a company may decide not to launch a product at all in a number of countries, if it is not deemed commercially worthwhile. ERP may also lead to price convergence, which can disadvantage countries with already low health budgets (and average patient income), which will pay a higher price than if they did not use reference pricing.¹²

Differential pricing has been put forward as an alternative strategy, both by the pharmaceutical industry and some other stakeholders.¹³ 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 proposed that value-based pricing may help balance short-term cost effectiveness with long-term incentives

ⁱ Also known as “external reference pricing or international price comparison / benchmarking, is defined as the practice of using the price(s) of a medicine in one or several countries in order to derive a benchmark or reference price for the purposes of setting or negotiating the price of a medicine in a given country.” It is usually limited to specific medicines, such as originator, prescription-only or new medicines. (Study on enhanced cross-country coordination in the area of pharmaceutical product pricing. Final report, December 2015; page xiv.)

for industry to develop added-value products. EPF is contributing to reflections on health outcomes, together with other stakeholders.¹⁴

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 determined and the different factors applied in assessment are very different in different OECD (and EU) countries. There is also no commonly accepted definition of value.¹⁵

EPF believes that future approaches towards pricing and reimbursement will need to be linked to the (added) value of a medicine for patients, and to involve some degree of differentiation between countries. We believe the constructive discussion at European level must 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**.ⁱⁱ 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 and may lead to reduced access. EPF believes that Member States should prioritise the interests of patients’ and public health over the internal market, and take action to limit the negative impacts of parallel trade where necessary. Member States would have to agree to limit external referencing for those medicines where a differential strategy would be adopted; as well as address other possible practical issues.

Transparency of prices: proceed with caution

Many are concerned that national decisions taken on pricing and reimbursement are hampered by lack of transparency on the prices of medicines and the cost of research and development. The argument in favour of **transparency on medicines prices** is that it would make medicines pricing more realistic and more equitable. Essentially, that external reference pricing would work if Member States could know the actual prices paid by other Member States.

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”. The EURIPID EU-funded project has set up a price database for competent authorities, but until now it only

ⁱⁱ Parallel trade is also called parallel export; it happens when a medicine originally sold under patent protection “is traded in another country without control or permission from the original patent holder ... From a legal perspective, medicines as such are no exception to the free mobility of goods in the internal market.” Thus medicines from lower-priced countries are exported to higher-priced countries. (Study on enhanced cross-country coordination in the area of pharmaceutical product pricing. Final report, December 2015; page xvii)

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.¹⁶ Views between Member States vary.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 – e.g. in terms of undermining price differentiation – in the long-term.

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. Quite often, patients do not even know how to engage in the process.

Patients' engagement is in our view vital – both from a moral perspective, because the decisions directly impact patients' 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.

Assessing the true value of innovation

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?

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 the degree to which the medicine makes a tangible (positive) difference for patients and the nature that benefit.

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.

First, 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 Second, many products come with high uncertainty at the time of marketing authorisation – i.e., with evidence hinting at the value the product may have, but with a lack of sufficiently comprehensive data to completely back up that estimation. Therefore, a given product may not be in a position to have “demonstrated” added value at the time of marketing authorisation but may well demonstrate this over time, thanks to the ongoing collection of real-world data.

The importance of **incorporating the patient perspective in HTA** is increasingly recognised,²⁴ and the integration of patient-reported and patient-relevant outcome measures (PROMs) including quality of life, 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.²⁵

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.

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, even for medicines that do represent valuable innovation, if this makes the innovative medicine unaffordable and inaccessible.

Adapting the system to new scientific and technological realities

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. Personalised or stratified medicines, combinations, borderline productsⁱⁱⁱ, and advanced therapies will require new ways of evaluation. They will also need new ways of managing clinical use; even when an initial assessment shows a positive benefit-risk, it is very likely that additional data will need to be collected over time.²⁶

The European Commission recently set up an expert group on Safe and Timely Access to Medicines for Patients (STAMP)²⁷, 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 of improving patients' access to medicine, in close collaboration with the European Medicines Agency.

New concepts that are being explored include that of **adaptive pathways**.^{iv} This complex approach 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.²⁸

A new, collaborative mind-set, involving **early dialogue between all of the stakeholders** at EU level is needed from the earliest stages of medicines research and development, building on existing examples such as the SEED and MOCA initiatives.²⁹ The mechanisms ensuring input from patients must be expanded and strengthened, also nationally. This will help identify anticipated benefits and value, outcomes for patients. An early dialogue will also result in a 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 to ensure public health needs are given due attention.

Systematic collection of real-world evidence is the key to the success of value-based pricing approaches: robust systems must be in place for post-market data collection (on ADRs, changes to the benefit-risk balance, health outcomes in different patient groups). 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 system needs to ensure

ⁱⁱⁱ Products where it is not clear if they fall under the definition of medicinal product or some other category (for example medical device or food supplement) for regulatory purposes.

^{iv} 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.

companies' compliance with their data-collection obligations; the information collected must then be made quickly available for appropriate action to be taken by regulators, industry, medical professionals and patients.

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 are collectively referred to as **managed entry agreements** (MEAs). These 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 where the company is obliged to provide additional data on real-life performance of a medicine. They all have the common aim of facilitating access to new medicines in a context of uncertainty and high prices.³⁰

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 benefits 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 as they are aimed at managing uncertainties and at ensuring that the right patients benefit from the treatments.

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 scaling-up of pilots on early dialogue and to establish a "table for price negotiation" with a group of Member States, i.e., to take a collaborative European approach to negotiating the prices of medicines with pharmaceutical companies, rather than one that is fragmented.³¹ 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.³² 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 medicines³³ in which other Member States may be joining. Similar discussions have started between Bulgaria and Romania.³⁴

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.

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 and what constitutes a “fair” return on investment; such a debate implies the need for more transparency about the real costs of medicines’ research and development. Patients and the public/citizens 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.

Currently commercial companies, whose priorities are driven primarily by shareholder value, are 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.³⁵ 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.³⁶ 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.³⁷

In addition, other issues that need to be addressed to improve cost-effectiveness of care and outcomes for patients and society include: reducing waste in health systems; tackling over-treatment and the use of inappropriate therapies or services, as well as under-treatment; improving patient adherence;³⁸ fighting corruption, which is an important access barrier in some Member States; and counteracting and preventing unethical practices by some companies, which should be monitored and strictly sanctioned.

At the end of the day, investment in health is a political choice. Ensuring universal access to new, innovative medicines for all those who need it is a political choice. We urge decision-makers to reject the “zero-sum-game” approach, whereby health budgets are regarded as fixed and immutable, and take positive action to realise an inclusive society that values health and makes it a political priority.

We are convinced that investment in valuable innovation will result in a virtuous circle, whereby better health outcomes will eventually contribute to balancing the short-term financial impact of the investment, and possibly even generate greater efficiencies and savings in the entire

economy in the long term. We therefore call on European decision-makers and stakeholders to put patients' health first and ensure EPF's core principles on value and pricing of innovative medicines are applied to the fullest extent possible. EPF and our members will continue to play a constructive role in helping to achieve this.

Core Principles on Value and Pricing

Health and access to innovative medicines

1. Health is a fundamental right *as well as* a critical investment in the well-being, economic development and cohesiveness of society.
2. Medicines are not a consumer good like any other; patients are not merely consumers, and patients' lives cannot be measured in purely economic terms. Medicines are an essential public good and a core element of health policy.
3. Pharmaceutical companies should price new medicines responsibly to ensure that they are accessible and affordable. Pricing should consider *inter alia* a country's relative capacity to pay; budget impact; the extent of public funding that contributed to the development of a medicine; and the need to ensure universal access.

The centrality of patients

4. 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, patient-relevant clinical endpoints, and patients' views on benefits and risks.
5. Patients should be recognised as the most important stakeholder group in medicines pricing and value assessment, and the patient perspective should be at the heart of every assessment.
6. Frameworks, structures and methodologies should be developed for meaningfully incorporating patient evidence at all stages, from early dialogue to Health Technology Assessments, relative effectiveness assessments, and pricing and reimbursement decisions taken at national level.

Value, pricing and reimbursement

7. A coherent framework for "fair access" to innovative medicines that maximises societal benefit and patient access whilst avoiding unacceptable impact on healthcare budgets should be developed at EU level, through a consultative process led by governments with the participation of all stakeholders including patients. Such a framework should encompass at least the following elements:
 - Closer collaboration by Member States on price negotiations and scaling-up of pilots on early dialogue such as MoCA and SEED;
 - Adoption of common principles and mechanisms for encouraging and rewarding innovation in order to encourage continued investment in R&D;
 - Exploration of the potential of adaptive pathways, managed entry agreements and other mechanisms for optimising access and determination of value;

- More thorough exploration of differential pricing mechanisms, barriers and potential solutions to dealing with practical issues such as parallel trade.
8. Pricing and reimbursement authorities should be transparent about their decisions, how these are made, what criteria are used, 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 and the public.

Beyond medicines

9. Patients' needs go beyond medicines and include other therapeutic options, social and community services and peer support. 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 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/
- ⁵ 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
- ⁶ See e.g., IMS Institute (2015) "The Role of Generic Medicines in Sustaining Healthcare Systems: A European Perspective."
- ⁷ "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
- ⁸ "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.
- ⁹ Article 3, Treaty on European Union
- ¹⁰ Council Conclusions on Common values and principles in European Union Health Systems (2006/C 146/01)
- ¹¹ 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
- ¹² EC study on pharmaceutical pricing. See reference 11.
- ¹³ "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
- ¹⁴ "Enhancing Value in European Health Systems. The Role of Outcomes Measurement". Multi-stakeholder consensus document (2016). Available at www.eu-patient.eu/globalassets/policy/patientsafety/value-of-health-consensus-document.pdf
- ¹⁵ Paris V and Belloni A (2013) "Value in pharmaceutical pricing", OECD health working papers, No. 63, OECD Publishing, pp. 11-14.
- ¹⁶ EFPIA position paper, principle 1. See reference 13.
- ¹⁷ Member State representatives interviewed in Politico, "Health ministers talk drug prices, information sharing" (18.04.2016), " France wants EU to work together on drugs, but not too closely" (18.04.2016).
- 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 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
- ²⁵ EPF survey: "Patient Involvement in Health Technology Assessment in Europe," 2013. Available at www.eu-patient.eu/whatwedo/Initiatives/
- ²⁶ Project ADAPT-SMART: <http://adaptsmart.eu/early-access-is-it-worth-it-2/>
- ²⁷ http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm
- ²⁸ <http://adaptsmart.eu/>
- ²⁹ Mechanism of Coordinated Access to Orphan Medicinal Products ([MoCA](#)); Scientific European Early Dialogue ([SEED](#))
- ³⁰ 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
- ³¹ www.eu-patient.eu/globalassets/alliance-building/eurordis-epf-letter_call-on-payers_may2015.pdf
- ³² EPF-EURORDIS joint letter of 18 May 2016. Available at www.eu-patient.eu/whatwedo/Policy/Pricing--Reimbursement/
- ³³ www.deblock.belgium.be/fr/grand-duchy-luxemburg-joins-belgium-netherlands-initiative-orphan-drugs
- ³⁴ <http://www.eiu.com/industry/article/723225456/teaming-up-for-pharma-bargains/2015-06-04>
- ³⁵ Kaplan W et al., (2013) Priority Medicines for Europe and the World. 2013 update. World Health Organisation, Chapter 6.
- ³⁶ Ibid., Chapter 8.1.
- ³⁷ For example the United Nations High – Level Panel on Access to Medicines. www.unsgaccessmeds.org
- ³⁸ 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

