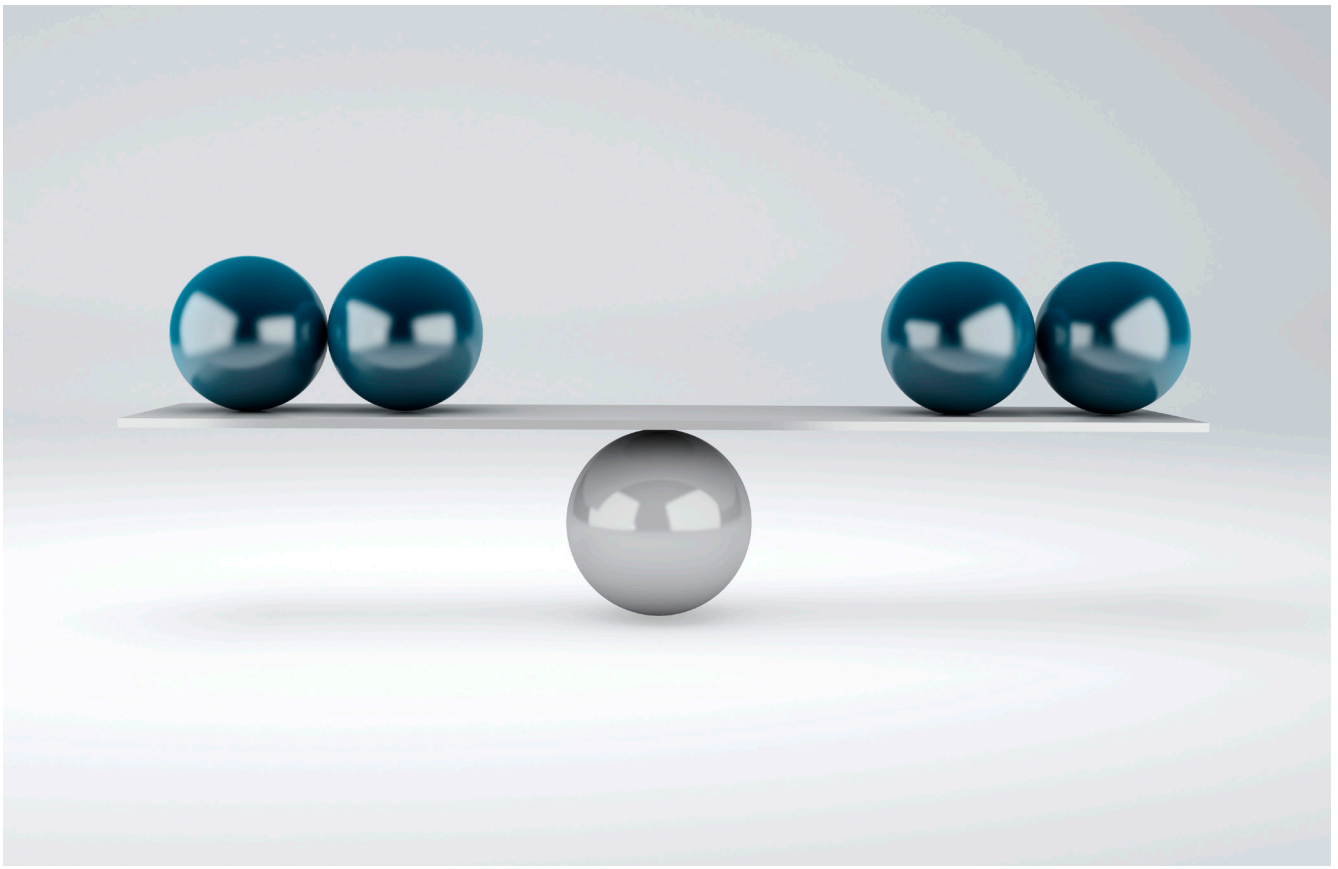
European Federation of Pharmaceutical Industries and Associations

A value-based approach to pricing

An EFPIA position paper

April 2023



Pharmaceutical innovation is key to healthcare in the 21st century

In recent decades, healthcare has changed beyond recognition. Pharmaceutical innovations have improved life expectancy and quality of life for patients around the world, whilst also easing pressure on healthcare systems and contributing to the overall productivity of society. However, healthcare systems in Europe are also facing demographic pressures. Older people are the main users of medicines and other healthcare services, and the share of the population aged 65 years and over is increasing in every EU member state. Pharmaceutical innovation has a critical role to play in helping healthcare systems adapt to this challenge. Breakthroughs in treatments for Alzheimer's disease, for example, could transform the expected trajectory of rapid increases in costs for dementia care in aging societies.

A value-based approach to pricing supports sustainable innovation

The goal of pricing of pharmaceutical innovations is to ensure that patients can access medicines in a way that is sustainable for healthcare systems, whilst also supporting a sustainable stream of innovation that delivers continuous improvements in the treatment options available for patients. Prices send signals to innovators about where to focus their R&D efforts, as well as determine the overall level of investment in health and expected value of innovation in the pipeline. This is why EFPIA is working with governments and stakeholders to identify and promote pricing approaches that ensure medicines are accessible, the healthcare system is sustainable, and the innovation pipeline is robust and focused on societal needs.

A value-based approach to pricing is based on the principle that prices should reflect the value of a new medicine to 1) patients, 2) health systems and 3) society versus the current standard of care. A value-based approach to pricing therefore means that healthcare systems appropriately reward innovation, and access to the most valuable innovations is prioritised. It also means that price signals are aligned with the patients' and citizens' priorities, such that the expected value of innovation for a given level of investment is maximised. A value-based approach to pricing also ensures that the level of investment in pharmaceuticals, and level of expected innovation in the pipeline, reflects their value to society.

Any other pricing approach is less efficient in signalling what society values, and therefore incentivizing the right kind and amount and quality of innovation. In setting a rationale, shared, framework for rewarding innovation, a value-based approach to pricing serves as a useful starting point for policies designed to address related challenges, including how to ensure countries contribute fairly to rewarding innovation.

There is significant heterogeneity in how countries in Europe have implemented value-based approaches to pricing

A value-based approach to pricing is achieved through first, assessment of the value of a novel medicine and second, determining prices based on that value. Therefore, a value-based approach requires both that value is comprehensively assessed and that the results of value assessment are reflected in pricing and reimbursement (P&R) decisions and, ultimately, net prices.

Many countries in Europe have implemented aspects of value-based approaches to pricing. At the same time, however, many dimensions of the value generated by medicines – such as helping patients to return to work or improving the health and quality of life of care givers – are not consistently recognised in value assessment frameworks.

In addition, some value elements may be recognised in HTA guidelines, but not considered in appraisals in practice, while some value elements are considered but only as part of 'additional' scenarios to be considered alongside the primary analysis-making it challenging to determine how much weight is given to them in appraisal. Finally, there is a lack of methodological guidance to facilitate inclusion of some value elements in in practice.

A value-based approach to pricing requires not only that value is comprehensively assessed, but that this value is then reflected in P&R decisions.

Research undertaken by the Office of Health Economics indicates that many European countries make some provision to reflect the results of value assessment in their P&R decisions. This might be as a clear, mechanistic link, as in the case of countries using explicit cost-effectiveness thresholds, or a more deliberative and/or qualitative approach may be used. We also note that a number of countries may implicitly recognise the value of treatments

for very rare diseases through an exemption for products receiving Orphan Medical Product (OMP) designation (when their budget impact is expected to be below specified thresholds). Demonstrating value can be challenging for treatments for very rare diseases, in part due to the small patient populations able to participate in clinical trials. The OMP exemption can ensure faster patient access to treatments in areas of high unmet need, where value is challenging to estimate but the potential to deliver value to society is high (and the budget impact is low).

However, most countries also make use of other pricing approaches or price control measures that disrupt the alignment between value and price. Several countries use external referencing, which is not only inconsistent with the principles of value-based approaches to pricing but may also be associated with patient access delays. Many countries also employ price control measures which further disrupt the alignment between value and price – meaning that many of the benefits of a value-based approach to pricing will not be realised. Several countries also employ further measures such as clawbacks (where innovators pay back revenue if spending on pharmaceuticals exceeds a certain threshold) to control the total pharmaceutical budget, which means that the investment incentives provided by a value-based approach are undermined or in some cases fully negated.

Furthermore, there are indications that the value societies place on health is underrecognized in spending on healthcare compared to other types of public expenditure. For example, there is evidence from some countries that sectors such as transport implement higher thresholds for life-saving interventions. Greater utilisation of novel medicines and investment in biopharmaceutical innovation are therefore expected to improve the welfare of patients and society.

Making a value-based approach pricing work in Europe for patients, healthcare systems, and innovations

We are putting forward a number of recommendations which can enhance how value-based pricing approaches are implemented, for the benefit of all stakeholders.

Embracing a value-based approach to pricing

Recommendation 1: fully embrace a value-based approach. Value-based approaches require that net prices are aligned with value, and the use of alternative pricing mechanisms disrupts this alignment. We recommend that countries should fully embrace a value-based approach, by avoiding the use of alternative pricing or cost-containment approaches.

Recommendation 2: extend a value-based approach to the indication level. An indication-based approach to pricing, where prices for the same medicine vary according to the value generated in treating different indications, refines the signals sent to innovators by facilitating an even more specific alignment between price and value. It also generates incentives for manufacturers to invest in developing novel medicines for all indications for which they are effective and safe. Whilst there are practical challenges to implementing value-based approaches at the indication level, there are also examples of how countries such as France and Italy have begun to operationalise some elements of an indication-based approach to pricing.

Enhancing value assessment and recognition

Recommendation 3: ensure meaningful involvement of stakeholders in value assessment and recognition. Involvement of stakeholders including patients, clinicians and carers in value assessment and recognition processes is crucial for ensuring that all perspectives on value of novel medicines are captured and appropriately integrated into valuation and access decisions. Involvement of developers at an early stage in the value assessment and recognition process is also important for facilitating discussion about which evidence should be collected during drug development, in order to facilitate a comprehensive assessment of value. Developers and value assessment bodies should invest in earlier and more frequent pre-launch cooperation, for example through developing joint scientific consultations.

Recommendation 4: develop a shared and holistic definition of value. Stakeholders should also work together at the country level to develop a joint, shared and holistic definition of value that recognises the multiple dimensions of value that novel medicines generate for patients, healthcare systems and society. This would help ensure that decisions on access to novel therapies are aligned with citizens' priorities and provide better guidance for future R&D efforts. It would also provide a starting point for collaboration between stakeholders on evidence and methods development, and for discussions about how the value generated by innovation could be shared to help healthcare systems navigate affordability and risk-management challenges.

Recommendation 5: enhance collaboration and share expertise across Member States. Fully embracing a fit-for-purpose system of joint clinical assessment at EU level (as introduced by the EU HTA Regulation) will allow for a more efficient use of resources and a more aligned view on the clinical value of a medicine across the EU, thus ensuring more capacity at national level for a differentiated and context specific analysis of the value of the medicine in respective societies. Appropriate resourcing of joint EU clinical assessment will be important to deliver on these benefits.

Recommendation 6: recognise qualitative evidence of value through deliberative processes. Recognition of qualitative evidence of value is important for progressing towards more comprehensive value assessment, and can provide concrete incentives for developers to make R&D decisions in line with a more holistic definition of value. Many value assessment bodies already recognise value qualitatively when quantitative evidence is lacking. Deliberative processes for value assessment, a form of structured-decision making, provide a mechanism for explicitly and rigorously assessing the value of novel medicines through the systematic consideration of heterogenous arguments and evidence, including qualitative evidence.

Complementing value-based approaches with other tools

Recommendation 7: use outcomes-based managed entry agreements to manage residual uncertainty. There are a number of payment models that can facilitate value-based approaches in the context of uncertainty about real world outcomes in clinical practice. Many countries already make use of outcomes-based managed entry agreements, and this could be extended through early dialogue between stakeholders to resolve challenges around defining (surrogate) outcome measures and measuring performance.

Recommendation 8: enhance data collection infrastructure to allow for iterative assessments of value post-launch. Holistic data collection and analysis across healthcare systems can enable more tailored assessments of value delivered (including for monitoring novel payment models and for managing uncertainty). Metrics for measuring different dimensions of value, including health outcomes for patients, should be standardised in order to increase comparability and reliability, building on the joint definitions of value developed by all stakeholders. Additionally, such visibility also allows manufacturers to better focus R&D efforts in areas where value for healthcare systems can be delivered better.

Recommendation 9: commit to Equity Based Tiered Pricing. A value-based approach to pricing can help to ensure that the level of investment in pharmaceutical innovation – and quality and quantity of the innovation pipeline – reflect the value of innovation to society. However, it does not solve the challenge of how investment in innovation should be distributed between countries, given there are incentives at the country-level to underinvest. EFPIA has therefore proposed a conceptual framework for 'Equity Based Tiered Pricing'. This builds on the principles of value-based approaches to pricing, with countries negotiating confidential value-based prices based on their own value assessments, but introduces a "best price rule", whereby innovators commit to ensure that less wealthy EU countries in the "lower tier" pay less than wealthier countries in the "upper tier".

Recommendation 10: promote competition. A value-based approach to pricing works in synergy with a competitive market for medicines, to deliver healthcare system sustainability. Indeed, value-based pricing itself promotes product competition; it increases the number of innovations which are expected in the therapeutic areas prioritised by society and, since rewards for innovations are in-line with the improvements they offer on existing alternatives (society will only pay for more value), innovators are incentivized to develop substantial improvements. There are additional, complementary tools available to policymakers for strengthening competition.

Conclusion

A value-based approach to pricing will help to deliver a sustainable stream of innovation that will benefit patients, healthcare systems, and payers. Whilst many countries in Europe recognise the potential of a value-based approach, and experiment with its implementation, there is significant heterogeneity in how far this has been done and to what extent other pricing approaches act to disrupt the alignment of value and price. There are many opportunities to improve how value-based pricing approaching is implemented, and EFPIA and its members are ready to work with Member States and all stakeholders to improve the systems in the interest of patient access, health system sustainability and future research and innovation in unmet health needs.

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