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European Federation of Pharmaceutical Industries and Associations

Policy Principles on Cross-country Collaborations on Medicines' Pricing and Access





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Executive Summary

EFPIA and its members support policies that deliver access to innovative treatments for patients, while ensuring the financial sustainability of our healthcare systems. In some cases this may be through cross-country collaborations, in others, patient access to medicines and healthcare system sustainability may be better served through national procedures.

EFPIA believes that a number of policy and structural issues should be addressed before cross-country collaborations truly deliver on their goals.

Four areas of activity common to the majority of cross-country collaborations have been identified and assessed in the context of developing the policy principles contained in this document. These are:

- Supra-national horizon scanning: Designed to support identifying, planning and managing the entry of new technologies into health systems, supra-national horizon scanning is a valuable complement to national horizon scanning processes and should aim at reducing duplication by providing national horizon scanning with high-quality information on the clinical properties of the technology. Industry can play an active role in developing these processes.

- Joint Clinical Assessment (JCA) and joint Health Technology Assessment (HTA): The multiplication of clinical assessments and HTA processes at European, national and regional levels creates risks of duplication of processes and of conflicting outcomes, ultimately resulting in potential access delays. This issue is all the more relevant and should be addressed upfront in the context of the future European HTA scheme currently under consideration: safeguards need to be put in place to ensure that the future system does not delay access as a result of duplication of the joint clinical assessment at the regional, national, or subnational level. Joint HTA should start with the establishment of clear criteria to accurately assess whether participating Member States have comparable healthcare systems and economies. If a company believes that participating in a cross-country collaboration pilot will delay or prevent access for patients then they should retain the right to introduce the medicine through the national procedure.

- Information sharing, purchasing and Joint pricing negotiations: EFPIA believes that joint pricing negotiations should have a long-term objective of broadening access for patients and stimulating the medical innovation that patients need. That means not using them solely for short-term, financial cost containment goals achieved through the negotiation of the lowest price. They should be based on solid legal grounds and offer legal predictability (such as on confidentiality of net prices and commercially sensitive information) to participating companies. The value of innovative medicines, measured through actual outcomes and benefits for patients rather than financial interest, should be the basis of pricing negotiations, including joint pricing negotiations.

- Joint public procurement: EFPIA recognises that joint public procurement is complex and it should only be used where it can improve access to patients to treatments. The use of joint public procurement should be proportionate to the needs identified by the participating Member States and limited to situations where purchase and supply of products cannot be ensured as efficiently by other means (e.g. individual purchase country by country). While in some circumstances joint public procurement may be an effective procuring tool for some categories of products, its use should not become a disincentive for innovation and company participation to joint public procurements should be voluntary in nature for the same reasons stated above.

Timely, efficient and sustainable access to innovative therapies is a fundamental goal that industry shares with patients, governments and authorities involved in cross-country collaborations. The implementation of cross-country collaborations is in its infancy, there is little successful experience of enhanced access driven by these initiatives. Until such evidence exists then national access processes will remain the most efficient tools to guarantee timely access for patients.

EFPIA Overarching principles

Industry supports initiatives that deliver the most timely access to medicines for patients. This may be through national processes or in some cases through cross-country collaborations. EFPIA believes that:

1. **Any Member States collaboration on pricing, reimbursement and access related issues should lead to broader and/or accelerated access for patients;**
2. **Since one of the aims of Member States' collaboration should be to accelerate patient access, the collective agreement should impose neither additional market access barriers nor additional price-related measures. Therefore, there should be no duplication between collective agreement and equivalent steps in participating countries;**
3. **Any voluntary Member States' collaboration on price should be confined to countries of similar economic and health-related needs;**
4. **Industry participation in any Member States' collaboration on pricing, reimbursement and access related issues should be voluntary; and**
5. **Any Member States' collaboration on pricing, reimbursement and access related issues should guarantee confidentiality of pricing and reimbursement agreements.**

1. Introduction

Over the last two years, European Member States have shown an increased willingness to work together on access to medicines. These initiatives such as BeNeLuxA-I, the Nordic collaborations or the Valletta Declaration, aim to improve the management of financial resources and/or enhance timely and efficient patient access to innovative therapies, two goals that EFPIA members support unequivocally.

Depending on circumstances there are instances, where cross-country collaborations could improve patient access to medicines and others where the optimal outcome will be reached through national procedure. EFPIA and its members support the option that delivers the most timely and effective access to innovative treatments. In addition, industry believes that, in the context of cross-country collaborations:

1. Any Member States collaboration on pricing, reimbursement and access related issues should lead to broader and/or accelerated access for patients;
2. Since one of the aims of Member States' collaboration should be to accelerate patient access, the collective agreement should impose neither additional market access barriers nor additional price-related measures. Therefore, there should be no duplication between collective agreement and equivalent steps in participating countries;
3. Any voluntary Member States' collaboration on price should be confined to countries of similar economic and health-related needs;
4. Industry participation in any Member States' collaboration on pricing, reimbursement and access related issues should be voluntary; and
5. Any Member States' collaboration on pricing, reimbursement and access related issues should guarantee confidentiality of pricing and reimbursement agreements.

For the purpose of this paper, EFPIA has categorised the main areas of focus of cross-country collaborations as follows: supra-national horizon scanning; Joint Clinical Assessment (JCA) and joint Health Technology Assessment (HTA); Information Sharing and Joint pricing negotiations; and Joint public procurement. Each category has been assessed against the principles mentioned above.

Industry's overall priority is that any cross-country collaborations contribute to better access for patients; they should therefore not create duplications – which would result in undue delays – neither with existing access policies and measures already in place in Member States participating to these initiatives, nor with upcoming EU activities, like EUnetHTA or those intended in the European Commission's HTA regulation proposal.

The implementation of cross-country collaboration is in its infancy, and to date there is little experience of enhanced access driven by these initiatives. EFPIA believes that a number of policy and structural issues should be addressed before cross-country collaborations truly deliver on their goals.

These include:

- The flexibility for companies to base their decision to participate in cross country collaborations on which approach will better support patient access to the innovative treatment. This may be through a cross-country collaboration or national level procedures.
- A guarantee that there will be no duplication of processes between the European, regional and national levels with the potential to result in delaying patient access (e.g. on JCA or HTA);

- A clear governance process guiding the creation and implementation of cross-country collaboration;
- A clear legal framework that guarantees legal certainty for companies who may want to be involved in pilot project and that ensures consistency with existing European and national legislations such as the Transparency Directive or the European rules on public procurement; and
- An approach to the design and implementation of cross-country collaborations that fully takes into consideration the expertise and input that industry and patient representatives can bring.

Some areas of cooperation, such as supra-national horizon scanning or the creation of joint patient registries, have a potential for synergies and efficiency gains across participating Member States. Timely and optimal access solutions entail a close reflection of the specific local socio-economic circumstances of each country and healthcare system. EFPIA believes that this can only be achieved by maintaining economic value assessments and pricing negotiations at national level: this approach provides for the necessary flexibility to take into account socio-economic contexts and healthcare system needs and agree on the optimal access solutions.

2. Supra-national horizon scanning

Horizon scanning is designed to gain actionable insight into innovative health technologies that will enter the market in the near future (usually 1 – 5 years). It involves gathering preliminary information about the medicine's clinical properties, expected benefits, and broader health system impact. Horizon scanning is often used to identify technologies which will be subject to a national HTA or managed entry process. Horizon scanning should be separate from budget forecasting, which is the process of forecasting the total budget impact of pharmaceuticals in a health system, including new market entries, generics and off-patent branded drugs. The horizon-scanning system should enable countries to map potentially important innovative medicines before they reach the market and develop their systems to timely assess which health technologies will be coming. Horizon scanning can help focus on the crucial interaction between industry and HTA to facilitate the uptake of possible disruptive innovations in healthcare systems in a sustainable way. Well-run horizon-scanning can therefore clearly improve access of patients to new treatments.

Horizon scanning conducted at a supra-national level in the context of cross-country collaborations can be a valuable complement to, and improve both efficiency and quality of, any national horizon scanning process.

Supra-national horizon scanning should aim to reduce duplication by providing national mechanisms with high-quality information on the clinical properties of the technology which is non-specific to the respective country or health system where the technology will be introduced. Examples of information elements which could be collected for the purpose of supra-national horizon scanning include International Non-Proprietary Name (INN), manufacturer, proposed indication, stage of development, general patient characteristics, comparators, regulatory status and expected date of regulatory approval.

The output of supra-national horizon scanning should not include a budget impact assessment. The budget impact of a new technology is dependent on factors which are country or even region specific, such as the organisational set-up and cost structure of the healthcare system, the size of the patient population and the price of the technology in that specific market. Examples of information elements that should be excluded from supra-national horizon scanning include epidemiology / burden of disease, availability of other treatments, current standard of care and/or treatment guidelines, expected price range, possible place in therapy, size of patient population.

The output of supra-national horizon scanning could include a top-line assessment of impact on healthcare systems in terms of organisation or service delivery (e.g. that the technology would enable shifting patients from in-patient to out-patient care, or would require early diagnosis of patients in specialist centres to be made effective). This assessment would have to be validated on national or regional level, where the detailed assessment in terms of impact on staffing and organisation would have to be made.

EFPIA advocates a clear and transparent mechanism for engagement with industry in supra-national horizon scanning. Companies are the primary source of information on new products and timelines before marketing authorisation, and they can validate and complement information collected from other sources.

Industry should contribute to the development of the process as it emerges. Specifically, this might result in:

- Proactively providing a set of data into the process in order to avoid duplication and unnecessary burden on companies;
- Keeping under review the data submitted, review and optimise the way the system operates, and commit to the timely input of data;
- Agreeing to a 'memorandum of understanding' on sharing information would be important as this will offer essential elements:
    - ◊ assurances about sharing information with the service;
    - ◊ confidentiality; and
    - ◊ alignment with the code of practice.

3. Joint Health Technology Assessment (HTA)

Joint Health Technology Assessments (HTA) are understood as including joint comparative economic assessments. HTA processes and policies already exist at Member State level and EFPIA believes that comparative economic assessments should remain a national competence as they include economic, ethical and legal factors which are country-specific and can vary significantly across Europe.

The European Commission recently proposed a regulation on HTA aiming at harmonising comparative clinical assessments across the EU, creating a system to conduct joint clinical assessments. The multiplication of clinical assessments and HTA processes at European, national and regional levels risks duplication of processes and conflicting outcomes resulting in potential access delays.

As a principle EFPIA supports a system that will streamline and guarantee the earliest possible patient access to valuable treatments. Irrespective of the scheme in place, safeguards should be provided to avoid any duplication of assessment between national and supra-national processes. Once the system outlined in the European Commission Proposal is in place there should be no duplication of the joint clinical assessment at the regional, national, or subnational level which delays access.

From the EFPIA perspective this means:

- Member States supporting joint clinical assessment in the context of cross-country collaborations should, as a prerequisite, embrace the European standard proposed for joint clinical assessment;

- On the long-term, the methodology used for regional joint clinical assessments will need to be aligned on the provision of the future European regulation on HTA. Refined methodologies should provide for a sufficient level of flexibility allowing an adequate management of evidential uncertainty in specific cases. Such flexibility should include the acceptance of the best available scientific evidence at the time of the submission, including, for example, data from case control studies, real world observational data, as well as the acceptance of indirect treatment comparison and surrogate endpoints;
- The creation of regional clinical assessment and HTA processes should not create additional technical hurdle and delays for national processes and a potential future European permanent framework;
- If clinical assessment and HTA processes are to be used in the context of cross-country collaborations, no process and outcome duplication with respect to existing or planned EU legislative proposal should be created, and swift access should be guaranteed;
- As a result, processes and outcomes of clinical assessments and HTA for a specific product in the context of cross-country collaborations should replace equivalent processes and outcomes at national level. Outcomes of cross-country processes should be mandatorily applicable for all participating Member States; and
- Clarity, legal certainty (including a right of appeal) and stakeholder involvement should be guaranteed so as to ensure trust in and efficiency of the collaborative process.

Comparative economic assessments should remain a national competence but, in cases where a cross-country collaboration is intent on conducting this type of assessment, at a minimum, the process should start with the establishment of clear and transparent criteria to accurately assess whether participating Member States have comparable economic, ethical and legal environments.

Industry participation to cross-country collaborations pilots and processes on joint clinical assessment and HTA should remain voluntary. Companies should be able to base their decision to participate or not on which route will ensure timely access to medicines for patients. Should a company be invited by Member States participating to a cross-country collaboration to take part in a regional JCA or HTA process and decline the invitation, the company should retain the right to engage at national level to support patient access. If a company chooses to engage in a regional JCA or HTA the confidentiality of commercially sensitive information contained in files provided to the authorities in the context of clinical assessments or HTA conducted in cross-country collaborations should be respected.

4. Information sharing and joint pricing negotiations

Information sharing is the process by which participating Member States share information about pharmaceutical policy, prices, suppliers and purchasing conditions - either bilaterally or in a structured way. Joint pricing negotiations the process by which participating Member States jointly negotiate with supplier(s) and agree to purchase from selected supplier(s) under common contracting conditions.

EFPIA believes that joint pricing[1] negotiations should have the long-term objective of broadening access for patients, as well as recognising and stimulating innovation and should only be used in this context. They should

[1] A large number of systemic and contextual elements can explain price variations across Member States. These include (non-exhaustively) economic context, such as the GDP per capita; burden of disease; different indications for which a product is reimbursed in a Member State; different reimbursement and price regulations, specific health system preferences and ability to pay; patterns of medicines usage; market structures; distribution and supply chain related components; hospital v. retail settings; and fiscal environment such as national clawbacks and mandatory rebates.

not be used for short-term, financial cost containment goals achieved through the negotiation of the lowest price which is detrimental to medical innovation in the long-term. Additional criteria such as the quality, value and benefits brought to patients are essential components of fair negotiations.

Procedurally, both information sharing and joint pricing negotiations should be based on solid legal grounds and offer legal predictability to companies who may decide to participate to these initiatives. Similarly, certainty should be provided on the confidentiality of net prices and all other commercially sensitive information such as clinical outcomes data.

EFPIA believes that information sharing can be beneficial when:

- Elements of products' clinical value are considered and recognised, such as clinical outcomes data, utilisation data and healthcare system performance data;
- Exchanges of information are made between Member States sharing similar healthcare conditions and systems, or, at a minimum, are interpreted in light of systemic and contextual differences;
- Prices and all other commercially sensitive information (such as non-public clinical outcomes data) shared during a negotiation process remain confidential. This information reflects specific conditions for patient access in a specific system and context that are not transferable to other situations. The respective price confidentiality rules applicable in each participating Member State and in individual contracts should be implemented;
- There is a guarantee that sharing of information among participating Member States will not lead to External Reference Pricing (ERP) which is a suboptimal way of ensuring appropriate and competitive price levels.

Regarding **joint pricing negotiation**, its use should be limited to situations where it will ultimately provide faster and/or broader patient access. The use of this procedure should be proportionate to the needs identified by the participating Member States and limited to situations where patients access to medicines cannot be ensured by other, more optimal and tailored means such as individual purchase country by country.

The value of innovative medicines, based on actual outcomes and benefits for patients and healthcare systems should be the basis of pricing negotiations, including joint pricing negotiations rather than a simple cost minimisation exercise. The development of outcome-based healthcare systems will increasingly deliver data and information to reward the options that deliver most value for patients, while contributing to the sustainability of healthcare systems.

In situations where different Member States wish to jointly negotiate on price, EFPIA recommends taking the following principles into consideration that would support effective and timely patient access:

- Joint pricing negotiations should replace equivalent domestic processes for decisions on pricing and reimbursement. Duplication of processes at national and/or regional level would result in a slower or more restricted access to new medicines. National processes should be adapted to directly integrate the outcome of supranational decisions into the national process, i.e. where the supra-national route is chosen, it should replace any domestic process;
- The legal framework applicable to joint pricing negotiations processes and the implementation of their outcomes should be clearly defined, particularly in relation to existing laws at national level. In line with

Transparency Directive provisions (Directive 89/105/EU) and EU antitrust legislation, the legal framework should provide for objective and verifiable criteria for decisions, clear timelines and the right of appeal. Because cross-country agreements might include access components (e.g. guaranteed reimbursement status in all countries or volume commitments for the defined groups of eligible patients) it is essential that participating Member States are able to fulfil these agreements on a local level (e.g. adapted reimbursement label, product formularies, treatment guidelines);

- Due to the wide variations of systems and context between Member States, joint pricing agreements may only be appropriate between Member States of similar economic and health-related needs. Systemic and contextual differences across participating Member States should be factored in the result of the negotiations. When several countries with for example varying levels of economic development try to determine or negotiate one single price, the resulting price will tend to be an average price that would likely disadvantage the least economically developed countries. As countries with the highest needs are at risk of being adversely impacted, EFPIA will not support collaboration among dissimilar countries unless there is a possibility for differential pricing between the countries;
- To support patient access, joint pricing negotiations should be voluntary for companies, meaning non-participating companies should not be penalised by a differential treatment at national level. Companies should remain free to engage at national level to support patient access, including in case of non-participation, or absence of agreement between the parties following a joint pricing negotiation process; and
- The terms of the agreement should remain confidential among the parties involved, in compliance of national rules and in line with the principle of confidentiality enshrined in a wide range of international legal instruments.

5. Joint Public Procurement

Joint public procurement can take different forms, ranging from coordinated procurement by multiple contracting authorities, each conducting a separate procurement procedure, to procurement where different contracting authorities jointly conduct one procurement procedure either by acting together or by entrusting one contracting authority with the management of the procurement procedure on behalf of the other authorities. Public procurement is regulated by Directive 2014/24/EU on public procurement and, at national level, by implementing laws.

In the context of cross-country collaborations, joint public procurement is complex and does not necessarily guarantee broader and timely access for patients. Contracting authorities from different Member States will have to agree on, for instance, the applicable process, applicable law taking into consideration the principles outlined below, language(s), and the distribution of roles and responsibilities among the participating authorities. Contracting authorities will also need to ensure that cross-country processes do not conflict with national rules and regulations such as pricing and reimbursement rules which are separate and different in scope and objectives. National processes should be adapted to directly integrate the outcome of supranational decisions into the national process, i.e. where the supra-national route is chosen, it should replace any domestic process.

In EFPIA's opinion, the use of joint public procurement should be limited to the situation where it will ultimately improve access to patients to treatments. It should be proportionate to the needs identified by the participating

Member States and limited to situation where access to medicines cannot be ensured as efficiently by other means (e.g. individual purchase country by country). Joint public procurement may for example be an effective procuring tool for open competitive segments (off-patent products), or vaccines, provided a number of conditions are fulfilled as outlined below. Most tendering practice might however prove inappropriate for novel innovative products, where there is limited clinical experience, e.g. first-in-class, unique mechanism of action, biological products with no bioequivalent alternative or products with proven incremental benefits.

For the future of health in Europe, the use of joint public procurement should not become a disincentive for medical innovation. To prevent this, the risks of aggregation of significant market demand and the commoditisation of medicines through joint public procurement should also be avoided.

In order to ensure broad and timely patient access to valuable medicines and at the same time to foster innovation through joint public procurement, EFPIA believes that the participating contracting authorities should follow the following principles:

- Guarantee long-term supply security and fair competition between all potential suppliers (in the case of joint procurement, the supply of medicinal products in non-participating countries should be ensured);
- Contain a variety of quality selection criteria, and price should not be the only determinant. Clinical input and review of the quality criteria should be encouraged;
- Clear technical criteria, bidding and selection processes should be established in compliance with national and European law; it should be fair, transparent and the governance clear to ensure legal certainty;
- Safeguard the principle of the autonomy of choice of the physician and the patient;
- Ensure a sufficiently broad choice of medicinal products, i.e. instead of a single medicine a variety of medicines should be available for physicians and patients in order to adequately match the variety of individual patient's needs;
- Allow continuation of treatment, i.e. any substitution/switching of treatment (particularly in the case of biologics) should only happen based on medical considerations by a treating physician; and
- Ensure that rules protecting company confidential information provided during the joint public procurement process as well as the agreed purchasing price are met.

To ensure timely patient access, company participation to joint public procurements should be voluntary in nature. Not participating in a joint public procurement process or in cases where no agreement is reached between the potential supplier(s) and the contracting authorities, suppliers shall always have the right to engage at national level to support timely patient access.

6. Conclusions

Timely, efficient and sustainable access to innovative therapies is a fundamental goal that industry shares with governments and authorities involved in cross-country collaborations. Cross-country collaborations should contribute to better access for patients and should not create duplication and delay.

The implementation of cross-country collaborations is not yet fully articulated and operative and to date, there is little evidence of enhanced access driven by these initiatives. To constructively contribute to this process EFPIA members have identified a number of policy and structural issues that will need to be addressed before cross-country collaborations truly deliver on their goals. In the absence of adequate measures to address these gaps, national access processes will remain the most efficient tools to guarantee timely access for patients.

EFPIA Brussels Office
Leopold Plaza Building, Rue du Trône 108,
B-1050 Brussels, Belgium
Tel: + 32 (0)2 626 25 66
www.efpia.eu * info@efpia.eu