



Access to innovative medicines

Joint position of the
European Social Insurance Platform (ESIP)
and
Association Internationale de la mutualité (AIM)

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About the *European Social Insurance Platform* (ESIP)

The *European Social Insurance Platform* (ESIP) represents over 40 national statutory social insurance organisations (covering approximately 240 million citizens) in 15 EU Member States and Switzerland, active in the field of health insurance, pensions, occupational disease and accident insurance, disability and rehabilitation, family benefits and unemployment insurance. The aims of ESIP and its members are to preserve high profile social security for Europe, to reinforce solidarity-based social insurance systems and to maintain European social protection quality. ESIP builds strategic alliances for developing common positions to influence the European debate and is a consultation forum for the European institutions and other multinational bodies active in the field of social security.

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About AIM

The Association Internationale de la Mutualité (AIM) is an international umbrella organisation of not-for-profit healthcare mutuals and health insurance funds in Europe and in the world which operate on the basis of solidarity. Currently, AIM's membership consists of 59 member organisation (including many national umbrella organisations) in 28 countries. In Europe alone they provide coverage of healthcare to around 200 million people and many more in Africa and Latin America. AIM strives via its network to make an active contribution to the preservation and improvement of access to health care for everyone.

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ESIP and AIM identify five priority fields for action to improve access to innovative medicines

1. Steering pharmaceuticals R&D on the basis of needs

- AIM and ESIP recognise the importance of pharmaceutical innovation for improving the health of European citizens.
- To maximize the benefits for patients and ensure access to innovative medicines within sustainable health systems, public and private investment in R&D should be steered towards public health needs such as those set out in the WHO Priority medicines report 2013. Public investment in R&D must be reflected in the price.
- Payers must be involved when setting EU and national R&D agendas and incentives.
- Different models for financing independent research e.g. by taxes should be explored.

2. Ensuring a central role for Health Technology Assessment (HTA) in market access and pricing and reimbursement decisions

- The development and use of transparent HTA tools and processes should be promoted at national and EU level to support Member States in their evidence-based pricing and reimbursement decisions.
- EU level cooperation on HTA should aim to foster the exchange of information and experience as well as re-use of joint work (assessment and data collection).
- We stress the need to measure and evaluate the benefits of EU cooperation at national level.

3. Strengthening national pricing and reimbursement mechanisms in an EU context

- ESIP and AIM call on the European Commission to continue to support exchanges with and between pricing and reimbursement bodies and stakeholders at EU level.
- In the interests of patients, payers and society, new medicines that do not demonstrate measurable benefits for patients should not be reimbursed. When added benefit is proven, reasonable pricing should be expected to enable access to all.
- In case of unmet need, potential flexible pricing and reimbursement models might be explored as part of a transparent and well-regulated access pathway.
- Voluntary cooperation in joint negotiations and procurement might also be foreseen.

4. Increasing transparency around innovative pharmaceuticals within the EU

- Greater transparency as regards clinical trials data, costs of R&D as well as pharmaceutical prices and expenditures is critical to facilitate better access to innovative high-quality and value-added medicines.

5. Supporting innovation in the context of sustainable health systems

- Additional mechanisms that help to preserve the sustainability of healthcare systems while improving patients' access to medicines should be supported e.g. promoting generic and biosimilar uptake, controlling anti-competitive behavior, maintaining the ban on direct-to consumer advertising.
- Further mechanisms to ensure the supply of medicines that have proven their added value for patients and health systems should be considered.

Introduction

Recent concerns regarding the very high prices demanded for some innovative medicines have highlighted the need for an EU-wide reflection on this issue. Indeed, the introduction of new innovative medicines presents serious challenges for the pharmaceutical sector, public health, health equality and the sustainability of healthcare systems. These four dimensions need to be taken into account when reflecting on a European strategy in the pharmaceutical area. Areas of conflict between them need to be carefully addressed in order to find the right balance between incentives for innovation, necessary to provide patients with high quality medicines, and the promotion of universal access to healthcare, where highly constrained health systems need to make efficiency gains to ensure financial sustainability and equal access for all.

ESIP and AIM welcome the attention paid to this topic by the Council in its Conclusions on “Innovation for the benefit of patients” adopted 1 December 2014. The Council proposes a number of actions to be undertaken by the Member States and the Commission in the framework of ongoing initiatives regarding EU cooperation on HTA, and cooperation within the Network of Competent Authorities for Pricing and Reimbursement (CAPR) and between the competent authorities and stakeholders (under the Process on Corporate Responsibility in the field of pharmaceuticals) aimed at facilitating exchange of information and collaboration in the field of pricing and reimbursement. These initiatives are actively supported by ESIP and AIM and provide the context for many of the recommendations below.

ESIP and AIM members have an important role to play in bringing solutions to the above challenges. Their involvement throughout the life cycle of medicines is essential to reduce the uncertainty for all stakeholders and to streamline the processes, improving patients’ access to innovative medicines with additional patient and health system relevant benefit. This paper highlights our views and concerns on this issue.

Two key principles however should always be preserved when developing policies in this field:

Firstly, patients are not simply consumers. One of the European Union’s central responsibilities is the protection of the health of European citizens (Article 168 of the Treaty on the Functioning of the European Union). Support for industrial competitiveness must not be allowed to supersede public health interests and pharmaceutical products cannot be viewed as ordinary consumer products.

Secondly, the central goal of each healthcare system in Europe is to ensure within those systems equal access to high quality healthcare for all. This mission of public interest should not be further threatened nor jeopardised by medicines with unjustifiably high prices.

As the field covered by access to innovative medicines is potentially very wide, we focus this paper on five priority fields for ESIP and AIM members: research and development, Health Technology Assessment (HTA), pricing and reimbursement mechanisms, transparency as regards research and development, marketing, prices and reimbursement of innovative pharmaceuticals and innovation in the context of sustainable health systems.

1. **Steering pharmaceuticals R&D on the basis of needs**

Investment in research and development (R&D) of pharmaceuticals is a precondition for the availability on the market of new technologies likely to save lives or improve health. In this area, ESIP and AIM consider that efforts should be made to increase the efficiency of the system in order to both maximise the benefits of research for patients according to their needs while contributing to the sustainability of healthcare systems. To date systems tend to steer investments to low volume, high price medicines (cancer and orphan drugs); other important areas are neglected.

Firstly, ESIP and AIM would like to stress the importance of **more actively involving the payers when setting research and development agendas and when defining incentives**, as they cover the costs of the treatments included in the scope of public health insurance systems. In this context, **we ask that national and EU-level research agendas take full account of the priorities set out in the WHO priority medicines for Europe and the world report 2013¹**, advised by a broad group of stakeholders under the Process on Corporate Responsibility in the field of pharmaceuticals. However we recall that the costs of clinical studies remain the financial responsibility of the pharmaceutical industry.

Public funding for research and development should be directed at evidence-based public health priorities such as those of the WHO priority medicines report with the aim of addressing unmet needs and/or burden of disease. Conditions should be set for delivery and use of the results, offering e.g. alternative rewards instead of patents. Aligning research priorities of the industry with those identified by public authorities may reduce the uncertainty for the industry, and facilitate and accelerate access to valued medicines. Indeed, a company is more likely to invest in the development of a therapy if it knows that public authorities responsible for the decisions on pricing and reimbursement and payers have already indirectly recognised its potential added value for patients and the health system.

However, **fostering innovation by rewarding research and development should not result in a situation in which a large part of the research is done using public funding while the profits are accrued privately by industry** selling the developed medicinal products to the same public. **Public involvement in research and development costs of pharmaceuticals must be reflected in their final price.**

Different models for financing independent research/studies carried out in the public interest or to avoid conflict of interest should be explored, for example through establishing a general tax² or a tax on the marketing expenditures of industry as exemplified by the programme on independent research on pharmaceuticals setup by the Italian Medicines Agency (AIFA)³.

¹ http://www.who.int/medicines/areas/priority_medicines/MasterDocJune28_FINAL_Web.pdf?ua=1

² J.E. Stiglitz & A. Jayadev. Medicine for tomorrow: some alternative proposals to promote socially beneficial research and development in pharmaceuticals. Journal of Generic Medicines (2010) 7, 217-226.

³ http://www.agenziafarmaco.gov.it/wscs_render_attachment_by_id/tipo_file0109.pdf?id=111.109018.1188484620191&language=IT&lenient=false/

2. Ensuring a central role for Health Technology Assessment (HTA) in both market access and pricing and reimbursement decisions

Outcomes of HTA processes do vary between the Member States. Thus, cooperation between Member States is needed to address this issue. It is important however that this cooperation should preserve the high standards of HTA that already exist in some Member States and take into account differences in national guidelines and differences in availability of therapeutic options.

The development of transparent HTA tools and processes should be further promoted and supported at national and EU level. They should aim to follow the life cycle of a technology from early dialogue, through short and long-term evidence generation, and reassessment. In all cases, the process of HTA should be separate and independent from that of market authorisation.

In ESIP and AIM's view cooperation on HTA at EU level can benefit both Member States and society by building capacity together. Realising these benefits however will depend on a number of factors that we identify below.

First, we think that **complete transparency is necessary** between HTA bodies as well as towards other stakeholders and the public to achieve acceptance and trust. This should include transparency as regards the data submitted by the manufacturer, and the quality assurance and management of the HTA products and processes within EUnetHTA. Trust in high quality HTA reports is a central precondition for a widespread national uptake and reuse of these assessments in Europe. Full transparency will also facilitate joint evidence generation and the sharing of expertise and information with countries with limited resources and where available data on a new technology is limited.

Transparency is also a precondition for the comparability of the data across Europe and the transferability of the results at national level. In this respect, **we welcome the efforts made by EUnetHTA to develop web-based tools for sharing information and enabling additional data collection over the short and long-term** (life data) by means for example of joint registries. In this context the organisation and funding of such registries will need to be addressed.

A sustainable public portal on HTA is useful for the sharing of standardised tools and templates developed by EUnetHTA. It will enhance the predictability of the system and help to streamline the assessment process. Using the public portal to provide teaching materials for both health providers and patients will also serve to enhance understanding and acceptance by users and prescribers.

Furthermore, **collaboration between HTA/appraisal bodies and industry** at both national and EU level **in the form of early dialogue**, such as piloted by EUnetHTA **can help to clarify expected value and required proof of value** in the early stages of development of a product, with the aim of facilitating and speeding up the assessment process following market authorisation. However, **such dialogue must remain voluntary and non-binding on both sides.**

Finally, **we welcome the efforts of the HTA network to facilitate the reuse of joint work** (e.g. common HTAs, Rapid Relative Effectiveness Assessments, guidelines on the form and content of submissions for HTA, methodology for cross-border assessment) in the national processes in order to maximise the benefits of the work both at EU and national levels. In this respect, **we would like to stress the importance of measuring and evaluating the benefits of EU cooperation on HTA.**

3. Strengthening national pricing and reimbursement mechanisms in an EU context

Currently the prices of new medicines are attaining levels which threaten the sustainability of healthcare systems. To address this issue, healthcare systems need to reflect on new, fair and affordable models developed in an open, “out of the box” mindset and involving all relevant stakeholders, with the triple aims of rewarding innovation that can prove added value for the patients, providing equitable access to patients across Europe and ensuring the long-term sustainability of healthcare systems

The fora for exchange of information between competent authorities for pricing and reimbursement (CAPR) and between these authorities and stakeholders (under the Corporate Responsibility in the field of Pharmaceuticals) facilitated by the European Commission DG Enterprise and Industry (now DG GROW) have in the past provided a safe harbour for discussions and exchanges in this field. **ESIP and AIM call on the European Commission to continue to support these exchanges in the development of their new strategy in the sector of pharmaceutical industry with the engagement of all relevant Directorate Generals.** ESIP and AIM want to be part of these discussions.

For ESIP and AIM, **reimbursing products which do not provide measurable benefits for the patients is unfair** both to society and to those companies that provide products with a proven value for patients and healthcare systems at a fair price. **Pricing and reimbursement procedures therefore need to set “evidence-based medicine” criteria** (e.g. demonstration of the added therapeutic benefit and/or economic value of the product over and above that of existing therapies).

Value-based pricing models have been proposed to reward innovation which can show real added value. However, **even when added value is indisputable, ethical (socially responsible) pricing should be expected from industry** in order to give patients in all European countries sustainable access to these drugs. This requires transparency as regards public and private investment in research and development (see 1) and transparency as regards the marketing strategies of industry, as well as budget impact/affordability considerations. In the absence of transparency, regulators should have the possibility of setting a maximum price. Here, close cooperation on an EU level could be useful.

However in many cases, medicinal products have market access without sufficient and adequate data concerning patient and health system related benefit, especially for medicines targeted at small populations (e.g. personalised medicines, orphan drugs) and cancer drugs. ESIP and AIM welcome the ongoing reflections at EU level to address this issue and in the framework of exchanges under the process of Corporate Responsibility in the Field of Pharmaceuticals, in particular relating to the method of coordinated access to orphan medicinal products (MoCA) and on managed entry agreements (MEA). Work to pilot potential solutions (e.g. the transparency value framework developed under MoCA) should be further supported by future strategies in the field of pharmaceuticals.

ESIP and AIM take note of the reflection at EU level on potential models of flexible pricing and reimbursement. For instance, **an adaptive pathway approach to pricing and reimbursement** involving early dialogue, conditional reimbursement and flexible pricing could be a way of sharing the economic risk between the company and the payer and allowing patient’s earlier access to promising new medicines. In ESIP’s and AIM’s opinion, **this approach is worth exploring but needs to be well regulated in order to minimise the risk to patients and to health systems**, to allow competent authorities to adapt the price of a pharmaceutical in response to new scientific developments in a

timely manner, **and to ensure complete transparency: commercial confidentiality should not be a condition of such models.**

In this context however, **restrictions on early access schemes should be upheld** to therapeutic areas in which no or only insufficiently effective alternative therapies are available. In these cases, high quality clinical studies (normally RCTs) are necessary after market access to ensure patient safety and optimal patient benefit.

Further, ESIP and AIM uphold the Council's notion that **any possible changes to Regulation (EC) 1394/2007 on advanced therapy medicinal products should not weaken the principle of quality, safety and efficacy** as the bases of marketing authorisation.

Furthermore, the AIM and ESIP consider that unjustifiably high prices should not exclude patients from access to innovative medicine. Possible solutions need to be explored using a "bottom-up approach" and in a "safe-harbour" environment where national interests can be openly debated and reconciled. In this context, **European countries could foresee the use of voluntary cooperation and tools for joint negotiations and joint procurement.**

Finally, we recall that **decisions taken as regards pricing and reimbursement are and should remain a competence of the Member States.**

4. Increasing transparency around innovative pharmaceuticals within the EU

Greater transparency of clinical data, the research and development costs of pharmaceuticals, and information on prices and expenditures would facilitate better access to innovative high quality and value-added medicines.

Transparency of clinical trials data, including those that do not lead to a marketing authorisation application and those targeted for commercial exploitation should be increased. **ESIP and AIM welcome EMA's efforts regarding its transparency policy and urge the enforcement of current reporting regulations** with regard to content as well as of timeliness and easy access. Early and full access to data by HTA and reimbursement bodies should streamline and speed up the assessment process post-authorisation, and facilitate post-marketing pharmacovigilance activities.

Increased transparency as regards research and development costs of pharmaceuticals (including public funding) **is also needed in order to foster responsible pricing.** Consideration should be given to the contribution from public funding, not only in basic research but also post marketing authorisation studies, when deliberating appropriate remuneration for the added value of new medicines.

ESIP and AIM also call on the European Commission to continue to support the exchange of information amongst pricing and reimbursement bodies under the CAPR network and amongst Members States on medicine prices and expenditures. Further, with the aim of providing greater transparency of prices across Europe we would urge the development of a reliable central information system for comparing actual prices and reimbursement levels based on the EURIPID project. This would help to ensure better access across Europe, where the negotiating power of different Member States regarding pricing is not equal.

5. Supporting innovation in the context of sustainable health systems

Any support for innovation needs to be balanced with measures aimed at ensuring the sustainability of healthcare systems, hence improving patients' access to medicines.

Therefore, **ESIP and AIM stress that the role of public health authorities in designing public health policies aimed at rationalising the constantly growing consumption of healthcare and health products (demand side measures) should be preserved.** Those measures can include the promotion of generic and biosimilar uptake, responsible prescribing, adherence and rational use. They should be supported by objective, independent and reliable information to patients and prescribers and exclude commercial interests.

In addition, **ESIP and AIM call on the European Commission to pursue its monitoring process of anti-competitive behaviour by companies, including patent settlements between originator and generic companies.** Indeed, settlements that delay generic market entry to the detriment of the European patients (whether legal or illegal with regard to Articles 101 and 102 Treaty on the Functioning of the European Union) and unresolved regulatory issues can lead to significant delays in patient access to both generic and innovative medicines and increased costs to already stretched healthcare budgets.

It is essential that Europe maintains its ban on **direct-to-consumer advertising of prescription only medicines by the manufacturers** (e.g. in the context of the Transatlantic Trade and Investment Partnership). Such advertising leads to increased costs for companies and healthcare systems without the benefit of objective, unbiased information for patients. In addition, reducing marketing budgets in favour of increased private investment in research and development would be in the public health interest.

Finally, **ESIP and AIM are of the opinion that mechanisms should be put in place to ensure the supply of medicines that have proven their added value for patients and health systems.** This might include sanctions on manufacturers that do not provide timely information on imminent unexpected stock-outs. **More importantly, mechanisms are needed to address the withdrawal of effective old molecules from the market purely for commercial reasons,** including to re-authorise the same molecule with a new indication at a much higher price (e.g. MabCampath® replaced by Lemtrada® for multiple sclerosis and chenodeoxycholic acid (Chenofalk®) replaced by Xenbilox® as an orphan drug).