EPF's feedback on the draft Implementing Act for Assessing and Managing Conflict of Interest

June 2024

The European Patients' Forum (EPF) welcomes the publication of the draft Implementing Act for assessing and managing conflicts of interest (CoI). This crucial document will determine which patients and clinical experts participate in Health Technology Assessment (HTA) cooperation at the EU level.

The experiences and perspectives of patients provide **essential evidence and guidance for evaluating the clinical effectiveness and safety of health technologies**, ensuring that HTA is conducted in the best interests of those directly impacted.

When it comes to HTA, it is essential to maintain the high integrity of the assessment process to secure trust and acceptance from national HTA bodies and involved stakeholders. Integrity implies accountability of those taking part, and transparency and reliability of sources informing the decision-making process. In other words, it is in the interest of all parties to generate soundly based decisions.

However, elevating the risk of CoI to an exclusion criterion for patient involvement in HTA risks producing the **unwanted effect of missing valuable input** generated through interactions with other stakeholders.

What are we calling for? Four recommendations to ensure optimal patient involvement in EU HTA processes

EPF calls for a **constructive approach** to CoI, based on **transparency,** which limits competing interests to the extent that they do not hinder patient involvement in the joint work and access to the best available expertise. Patients should not be penalised for being transparent and compliant with the mandatory disclosure of interests.

1. **Establish a scoring system to assess risk of Conflict of Interest among experts**

To avoid the mounting risk of lack of patient involvement, EPF calls on the Commission to adopt a more **granular approach** to assessing CoI. Specifically, we recommend establishing a **scoring system**[1] of CoI as a tool to measure the relative weight of activities that could generate a risk of CoI. Such a scoring system could be developed in collaboration with the HTA Stakeholder Network, including patient organisations and medical societies, to structure how and whether a conflict arises in a given context, based on a declared interest. A scoring system would provide clarity for all parties, track levels of risk with transparency and reduce arbitrariness in the selection of patient and clinical experts.

Such a tool would also enable patient organisations to identify whether diverse and balanced sources of unrestricted funding supporting the sustainability of patient organisations would create a risk of CoI in the context of the evaluation of a particular technology. Furthermore, it would allow patient organisations to self-assess whether certain behaviours or interactions with industry and other stakeholders may increase their score and therefore the risk of CoI of their leadership and members, thereby increasing the predictability of their ability to contribute to EU HTA and national processes.

Patient organisations often receive financial support from multiple sources, including pharmaceutical companies, but our empirical experience shows that this does not inherently bias their contributions. Diversity of unrestricted funding demonstrates that no single company can unduly influence the decision-making process of patient organisations, which are driven by governance structures composed of member patients and caregivers (in the case of national associations) and patients associations (in the case of

[1] Maharaj SV. A new method for scoring financial conflicts of interest. Int J Occup Environ Health. 2015;21(1):49-52.

national platforms and umbrella federations). Acknowledging the complexity of funding sources for patients will prevent the undue exclusion of valuable patient perspectives from the HTA process.

A scoring system would include a **scale of potential bias** to assess specific collaboration to weight the risk of conflict (i.e. general engagement with company representatives and their specific departments, discussions on a therapeutic area, or on a single product, type of funding source, purpose of funding) and allow a thorough assessment of the profiles avoiding the risk of subjective decisions by the European Commission.

In addition, we welcome the clause included in Art. 8 §5 of the text under review, allowing for a **derogation** from Art. 8 §1-4, applicable in case of the absence of patient and clinical experts with CoI. Nevertheless, we call for the adoption of the above-mentioned scoring system to ensure that the possibility of involving patient and clinical experts in the joint work is as **predictable** as possible, to ensure appropriate investment of capacity and readiness to participate in a given EU HTA process.

2. **Allow the "expert witness" status for experts in HTA process**

We also recommend the adoption of mitigation measures similar to the **'expert witness' status used by the European Medicines Agency (EMA)**[2]. This format allows patients with assessed risk of CoI to participate under certain conditions, e.g. when there are only a very limited number of patient representatives available, ensuring that their input is heard while maintaining the integrity of the assessment. This would allow patients to answer questions and participate in discussions without taking part in the assessments. Such flexibility ensures that the pool of patient representatives is not restricted and that their unique insights are incorporated into the HTA process.

3. **Develop guidelines to mitigate CoI among patients**

Mitigation measures should be transparent and clearly communicated to patients and patient organisations to ensure that they understand the procedures and safeguards in place.

We recommend that the European Commission develop a **set of guidelines** explaining the process and criteria for assessing CoI, mitigation measures and strategies implemented to manage CoI.

Clear guidelines on interpretation of the rules are also needed to ensure consistency. Not only do countries have different employment rules, but some terms, such as "comparator", which are part of the exclusions, are not properly defined. Typically, it is determined during the evaluation based on input from clinical experts, which may be contradictory. Therefore, a standardised approach to the definition of comparators should be developed to ensure consistency.

4. **Offer accessible and patient-friendly onboarding support for EU HTA processes**

We further recommend that processes and documents should be made more patient-friendly. Declaration of Interest (DOI) forms and guidelines should be clear and accessible to all patients across the board, and support should be provided to patients during the DOI process. Smaller organisations and individual patients may also face disproportionate challenges in complying with the administrative and procedural requirements, i.e. frequent updating of DOIs, compared to larger entities. Measures to support smaller entities and individual patients, such as streamlined processes or additional resources, should be considered by the HTA Coordination Group and delegated to the European Commission. Similar to the EMA, dedicated staff could be appointed by the European Commission to guide patients and clinicians through the entire cycle from enrolment to post-evaluation to ensure meaningful and smooth engagement.

Why are we asking for this? What generates a conflict of interest?

It is important to recognise that **patients are not involved in the HTA Coordination Group nor in the decision-making process** of the EU joint work on HTA. Relevant subgroups of the HTA Coordination Group

will decide whether to consider patient input. This mirrors the approach taken by the National Institute for Health and Care Excellence (NICE)[2], which recognises and weighs the different interests of the patients involved, but - as they are not decision-makers - patients are not prevented from contributing their expertise to NICE's HTAs. The conditions set out in the draft Implementing Act are in some cases even stricter than the policy adopted by the EMA, where patients sitting in scientific committees are entitled to voting rights[3].

We are concerned that some of the criteria defined in the Implementing Act could result in a situation where no patient would be able or willing to participate in the joint EU HTA work. This could significantly **limit patient involvement and impair the European Commission and Member States to ensure the best available patient knowledge participation in the assessment.**

For rare diseases, disease subtypes, or diseases with high mortality and morbidity, there is **a limited pool of available patients meeting the requirements for involvement** (e.g., European or international perspective, experience/expertise in the disease area under consideration, English speaking skills, absence of CoI). Many diseases have a significant impact on autonomy and cause severe disability and significant burden on quality of life, making it extremely difficult for patients living with these conditions, or their representatives, to bear the burden of such assessments on their own. The risk is that the above-mentioned package of criteria including also CoI ones, limited awareness and knowledge of HTA and lack of compensation, will further narrow the list of available patients, despite the necessity to ensure inclusivity, diversity and representativeness within the European patient community (in terms of age ranges, gender, culture etc).

In particular, patients who play a leading role in patient organisations will be excluded from participating in joint work on health technologies if the organisations they are affiliated with receive funding from companies with technologies under assessment. This restriction is particularly problematic in some disease areas where patient organisations are relatively small and composed mainly of leaders. In addition, some patient organisations receive unrestricted funds and grants from more than 40 industries, which in principle is a measure to mitigate and limit the risk of CoI. However, according to the draft Implementing Act on CoI, income fragmentation becomes a major barrier to participation as there is no clear reference to the volume of funding per industry, nor to the distinction between unrestricted grants and sponsorship, and paradoxically it would prevent patient leaders from bringing their legitimate and representative knowledge to EU HTA work.

The reality is that, due to the voluntary nature of advocacy, many patient experts receive direct or indirect funding from companies, which serves to cover, for example, their travel expenses. Patients cannot be expected to travel at their own expense and spend many days away from paid work to represent and advocate on behalf of patients.

Role of Patient Organisations

Patient organisations work with guiding principles and rigorous operating standards. Therefore, they are a key resource in managing CoI, as they can **facilitate transparency** and **raise awareness among their patient community**. Investing in building the capacity of these organisations and individual patients helps them understand conflict of interest policies. In turn, patient organisations can educate their community, promote transparency about their activities and funding, and enforce clear conflict-of-interest policies to manage potential conflicts appropriately[4]. Strong transparency policies build trust with HTA bodies and other stakeholders.

[2] See "How NICE manages the potential conflicts of interest of patient experts"
[3] This is the case for the EMA's Pharmacovigilance Risk Assessment Committee, the Committee for Orphan Medicinal Products, the Committee for Advanced Therapies and the Paediatric Committee.
[4] See EPF Transparency Guidelines.

With clear and balanced criteria - such as our proposal for a scoring system applied to patient organisations and individual patient experts - organisations can **diversify their workforce capabilities** to be able to engage with regulators/HTA bodies/payers and developers at the same time[5]. One strategy used by patient organisations to overcome potential CoI among their members is to set firewalls between different (i.e. informative, advisory, capacity building, fundraising) activities, although very small organisations may not have the capacity for such restructuring. In addition, patient organisations call for sustainable access to public funding, as this can help to diversify funding sources and support independent operations[6]. A diverse and well-supported pool of patient representatives ensures that patient input is based on a wide range of experiences and expertise, and free from undue influence by any single stakeholder.

Role of Other Stakeholders

As health technologies are developed for patients, they are central to engaging with all stakeholders throughout the development process and beyond. Patients' involvement in developing these technologies means they often interact with multiple stakeholders simultaneously and over time, creating potential CoI. Understanding how interactions with one stakeholder may affect a patient's ability to engage with other sectors in the ecosystem is essential to anticipate and manage such situations appropriately.

This responsibility places a significant burden on patients to understand the ethical and legal implications of their involvement and to keep and disclose accurate records of their interests and activities. Stakeholders who work with patients **must inform them of the implications** of these activities. In addition, stakeholders must be **transparent about how they use patient input**, and clearly delineate the scope and impact of the input. This transparency is particularly important as the draft Implementing Act prohibits patients from participating in joint work related to health technologies of a company that has paid or reimbursed them for activities where the therapeutic area cannot be identified.

We urge the European Commission and the HTA Coordination Group to consider these recommendations in order to ensure a balanced approach to the management of conflicts of interest, facilitating meaningful patient involvement while maintaining the transparency and integrity of HTA at EU level.

ABOUT EPF

EPF is an umbrella organisation of patient organisations across Europe and across disease areas. Our 79 members include disease-specific patient groups active at EU level and national coalitions of patients representing 19 countries and an estimated 150 million patients across Europe. www.eu-patient.eu

For all media inquiries please contact EPF Communication Manager Flavia Topan at flavia.topan@eupatient.eu

Co-funded by the European Union

Funded by the European Union. Views and opinions expressed are however those of the author(s) only and do not necessarily reflect those of the European Union or HADEA. Neither the European Union nor the granting authority can be held responsible for them.

[5] See PARADIGM Code of Conduct for all stakeholders involved in patient engagement activities within medicines development.

[6] See EU4Health Civil Society Alliance statement, May 7th 2024