EPF's feedback to the Implementing Act on HTA cooperation with the EMA

July 2024

The European Patients' Forum (EPF) welcomes the publication of the draft Implementing Act on Health Technology Assessment (HTA) cooperation with the European Medicines Agency (EMA).

We strongly support **close interaction between regulators and HTA bodies** to enable faster patient access to medicines. Although the EMA's assessment is distinct from the HTA one carried out at EU level, the two processes are closely interlinked and affect the entire medicine lifecycle, including determining whether a medicine provides significant benefit.

A key aspect of this interconnection is the **involvement of patients**. The EMA has a long history of patient engagement, supported by established frameworks such as the Public and Stakeholder Engagement Unit within the Stakeholder and Communications Division. The EMA's good practice on patient involvement should be an integral part of the HTA collaboration with the EMA to build the capacity of the HTA bodies to fully embed patient involvement in the joint work. Efforts should be made to align patient involvement practices, especially regarding the confidentiality of profiles[1] and information[2]. Confidentiality policies need to be clarified and communicated to patients, particularly when the same patient is involved in both the EMA and the JCA, to ensure compliance and prevent any discrepancies.

The draft Implementing Act further allows for the same patient to participate in both the EMA and HTA processes. This dual involvement enhances the contribution of patients, as their participation in the EMA's Protocol Assistance and Scientific Advice meetings – where they discuss comparators, treatment outcomes and trial design – prepares them to contribute effectively to the scoping and Joint Clinical Assessments (JCAs).

However, participating in both processes can be burdensome for patients, given the simultaneous demands of preparing for EMA and HTA procedures, such as overlapping meetings and extensive documentation to review. While patient experts will be remunerated at the EMA, no remuneration is planned for patients involved in HTA at EU level, which may exclude individuals from lower socio-economic backgrounds. Having the same patient involved in both assessments runs multiple risks: tokenism; always having the same people involved, and therefore reducing the representativeness of the community and the opportunity for robustness of input. The representativeness issue is even amplified in case only one patient is invited to contribute to the Joint Scientific Consultations (JSCs) or JCAs.

To address these potential issues, we suggest diversifying the workforce and ensuring that different patients are involved in the EMA and JCA processes. Widening the pool of patients and diversifying their recruitment sources is essential to: 1) ensure comprehensive and diverse patient input into all assessments, and 2) reduce the risk of generating biases or potential conflicts of interest. Patient organisations are a key resource in widening the pool of patients and identifying the relevant profiles. They

[1] The EMA publishes data on patient experts involved in its Scientific Committees. See the Committee for Orphan Medicinal Products as an example.

[2] The EMA publishes a summary of the opinions of the EMA's scientific committees at the end of the evaluation process and all clinical trial data for medicines with new active substances that received a CHMP opinion or were withdrawn before the opinion stage from September 2023.

can raise awareness within their communities and support the development of the different profiles. With clear and balanced conflict of interest criteria, they can diversify their workforce capabilities to ensure that their communities can engage with regulators and HTA bodies.

Through their networks and established governance and transparency mechanisms[3], patient organisations can also better identify appropriate profiles and coordinate the engagement, in particular providing support to identified patients who are not trained or have no previous experience of participating. Patient organisations should be aware of the planning and forecasting of JCAs and JSCs as early as possible. This will allow them to plan ahead, identify appropriate experts and train them if necessary.

Engaging in meaningful dialogue with the patient community will be essential to ensure that the implementation of the EU HTA Regulation meets the needs of patients. EPF is committed to being a strong and constructive partner in the implementation process and will continue to engage with the European Commission, Member States and key stakeholders.

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@eu-patient.eu

[3] See EPF Transparency Guidelines.