10 Key Recommendations from Patient Organisations on Joint Clinical Assessments under the EU HTA Regulation

June 2024

Patient organisations, European patient organisations and national umbrella patient organisations have actively participated in the European Commission's consultation on Joint Clinical Assessments (JCA) of medicinal products. Drawing on their practical experience and expertise in patient involvement, they have made recommendations towards an effective patient involvement in JCAs, one that serves to provide evidence on patients' experiences of living with the disease, navigating treatments and assessing the potential impact on health technologies on quality of life. The **following ten points summarise their input and we highly encourage they are considered when developing guidelines for JCAs** to ensure meaningful and practical patient involvement:

1. Establish a **predictable framework** for patient involvement in JCAs
2. Include **input from patients, carers and patient organisations**
3. Include **patient experience data** in JCAs
4. **Streamline patient involvement** throughout the process
5. Provide **plain language summaries** of technologies
6. **Broaden the pool of patients** and specify selection criteria
7. Provide **support to patients**
8. Make JCA and summary reports **available in all EU languages**
9. Provide **feedback** to patients
10. Adopt **a constructive approach to confidentiality and conflict of interest**

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1. Establish a predictable framework for patient involvement in JCAs

A clear and certain framework will ensure that patients and their representatives are adequately prepared and resourced to contribute to joint assessments. In particular, we request a clear definition of patient involvement, including the relevant timeframe and type of content and format expected for their contribution. In general, patients should be permitted to provide both written and oral input, following the standard practice developed by the European Medicines Agency (EMA) and Health Technology Assessment (HTA) bodies worldwide.

Furthermore, the EMA and JCA processes will be conducted in parallel, with both entities recruiting patients simultaneously and potentially contacting the same individuals. It is necessary to determine whether the same patient can participate in both processes, as it may be the only viable option in certain cases (i.e. rare diseases).

2. Include input from patients, carers and patient organisations

Patient organisations provide a unique, aggregated view of the patient community, offering robust and evidence-based information to the process. An evidence-based narrative is the most effective way to accurately capture the burden of disease and the added value brought by a certain innovation in terms of available alternatives, especially for diseases where symptoms and their impact vary greatly. Furthermore, in the case of rare diseases or diseases with low survival rates, it may not always be possible to find an individual expert patient volunteering for the process.

In the same way as the health technology developer, patient organisations should be able to submit, on their own initiative, new relevant information, data, analyses and other evidence to the Coordination Group in cases where the JCA report did not specify the need for an update. On the basis

of such new information, the Coordination Group may decide to include an update in its annual work programme.

In addition to patients and their organisations, carers should also be able to contribute to JCAs. For certain patient populations where effective patient participation in the procedure is not always possible, including paediatric patients, elderly populations or people with disabilities, it is crucial to have the lived experience of the disease integrated in the assessment process through the involvement of the caregivers. Furthermore, the references to unmet needs and impact on quality of life on certain diseases and populations that develop dependency, should be also enriched and assessed from a caregiver perspective.

3. Include patient experience data in JCAs

Patient experience data[1], such as core outcome sets and patient preference studies, co-designed and co-developed with patients, should ideally be incorporated to complement any type of patient involvement. This approach enables the collection of robust evidence at an earlier stage, thereby promoting a broader perspective across disease severity, age, gender and place of residence. It also ensures that the patient perspective is incorporated into the HTA process, even when time and resources are limited.

The EMA's ongoing work on the generation, collection and use of patient experience data for regulatory purposes, together with the support of the European Medicines Regulatory Network[2], is a crucial step forward. These advancements need to be reflected in joint assessments at EU level to include representative patient input.

4. Streamline patient involvement throughout the process

It is important to involve patients from the start of the process, and not just when the assessment scope and draft JCA and summary reports have been already produced. Seeking patient input after document consolidation risks tokenistic patient involvement, as their insights, if incorporated at all, would be included too late in the process.

Therefore, patients should be involved in the preparatory phase to provide input into the initial development of the assessment scope and the JCA and summary reports. They should have the opportunity to contribute at every stage, including the formulation of the PICO (population, intervention, comparison, outcome) elements, particularly on patient-relevant outcomes and endpoints.

5. Provide plain language summaries of technologies under assessment

Patients need to have access to timely, clear and accessible information about the technology they are being asked to comment on. In addition to the full JCA dossier submitted by the health technology developer, which must be accessible to the relevant patient experts and consulted patient organisations, plain-language summaries are useful not only for patients and patient organisations, but also for all contributors who may not have specific expertise in the condition or technology being assessed.

As a model of best practice, Health Technology Assessment International (HTAi) has developed the Summary Information for Patients (SIP) template[3]. The SIP has been developed based on a model used by the Scottish Medicines Consortium, with feedback from multiple stakeholders (including patient

[1] See EMA workshop on patient experience data in EU medicines development and regulatory decision-making workshop.

[2] European Medicine Agencies Network Strategy to 2025 (Europa.eu) ; Regulatory Science Strategy to 2025 (Europa.eu)

[3] See section on Patient Participation.

organisations and HTA bodies) and is designed to be adapted by any global HTA body to support effective patient involvement. The SIP has also been successfully piloted by the National Institute for Health and Care Excellence in England and the Pharmaceutical Benefits Advisory Committee in Australia.

6. Broaden the pool of patients and specify selection criteria

The list of resources proposed by the European Commission to identify relevant patients may inadvertently exclude patients with low health literacy and/or from disadvantaged socio-economic backgrounds, as it assumes that patients should already be aware of European or national processes and involved in existing initiatives. This approach can result in a highly exclusive process dominated by gatekeepers for patient involvement. The pool of patients should be widened, for example by including the EU Health Policy Platform in the list of databases or by organising an open call for volunteers to register online. National patient organisations and national HTA agencies could also assist in the dissemination of information ensuring a more inclusive approach.

In addition, it is essential to develop, in collaboration with patient organisations, inclusive, transparent and consistent criteria to ensure representativeness of the patient community. Criteria covering multiple EU Member States should not exclude competent patients with national expertise or over-represent certain EU nationalities.

7. Support patient participation in JCAs

Patients need to be supported throughout their involvement in JCAs to ensure meaningful participation and to avoid burdening patients who are often in vulnerable situations. Investment in dedicated patient engagement staff to facilitate recruitment and ongoing support is essential. For example, the Public and Stakeholder Engagement Unit of the EMA's Stakeholder and Communications Division assists with recruitment, answers questions, explains the process and anticipates questions. Patients should receive comprehensive onboarding on the administrative procedures, including timelines and stages of the process. Patients also need support as the issues can be complex and emotional for them. In such cases, patient organisations can provide training and support to patients, helping them to prepare for the questions and issues they may face. Confidentiality concerns should not hinder this support, as is the case with the EMA (see point 10).

In addition, patients should be compensated for their time and effort to enable participation by those from lower socio-economic backgrounds.

8. Make JCA and summary reports available in all EU languages

Given the European scope of the JCA, the summary reports must be published in all official EU languages to ensure adequate access to information for all EU citizens, regardless of their language. The provision of translations is essential for promoting health equity, as it enables patients, carers and patient organisations across Europe to fully understand and engage with the assessment reports.

The lack of translation of the summary report can exacerbate inequalities in access to healthcare and health information. Translations further support national health authorities and patient organisations in their local dissemination efforts, facilitating more effective communication at the national level.

9. Provide feedback to patients on their participation in JCAs

Patients should be given clear, concise, and understandable feedback on how their input has been used and what could be done in future stages of their involvement in JCAs to ensure continuous improvement in the assessment process. This could involve guiding patients on how to provide more effective input or highlighting areas where their insights could be particularly valuable in upcoming assessments.

Implementing a robust feedback mechanism ensures that patients feel that their involvement is valued and meaningful, which increases their motivation to participate in future assessments and fosters a collaborative environment.

10. Adopt a constructive approach to confidentiality and conflict of interest

The implementation of JCAs should ensure consistency with the upcoming Implementing Act on conflict of interest and adopt a constructive approach to both confidentiality and conflict of interest.

Patients involved in the study may wish to involve other expert patients of a particular population, gender or age and/or may need to discuss some aspects of the JCA with members of their patient association – when they are part of any – to provide a more informed opinion. However, this could be hindered by the confidentiality agreement, which prevents communication with other patients. It is therefore crucial that confidentiality agreements consider such needs and that the advice of expert patients to involve others is respected, reviewed and followed by the assessor.

Furthermore, a constructive approach to conflict of interest based on transparency should be taken, limiting competing interests to the extent that they do not hinder patient involvement in JCAs and access to the best available expertise.

In some cases, flexibility should be allowed, for example, for rare cancers or diseases with high mortality or morbidity. For instance, if a patient cannot be identified due to a conflict of interest, a special status of expert witness (on behalf of the patients) should be created. This would permit expert witnesses to answer questions and join in discussions without participating in the assessments.

SIGNATORIES:

CANCER PATIENTS EUROPE

EFA
European Federation of Allergy and Airways Diseases Patients' Associations

EHC
european haemophilia consortium

EKPF
EUROPEAN KIDNEY PATIENTS' FEDERATION

emsp

EPF European Patients Forum

EU-PFF
EUROPEAN PULMONARY FIBROSIS FEDERATION

France Assos Santé
La voix des usagers

LYMPHOMA COALITION

MPE
Myeloma Patients Europe

THALASSAEMIA INTERNATIONAL FEDERATION