executive insight
HEALTHCARE CONSULTANTS

EU's new Joint Clinical Assessment (JCA): will patients have a say?

The EU's new Joint Clinical Assessment will kick off in 2025 aiming to broaden and accelerate patient access to innovative medicines. Patient consultation is part of this process, but patients and patient organizations will need time to optimally prepare to ensure their voice will be heard.

Patient access to medication remains a global and multifaceted issue. Access impacts common chronic conditions, such as diabetes or heart disease, as well as rare diseases, adding to the burden experienced by patients, their caregivers, and the healthcare system at large.

Barriers to access – be they affordability, reimbursement challenges, access delays, access inequities or limited knowledge with regards to disease and treatment – need to be understood in the context of the patient and the role they play in their own care.

Patient access inequalities in the EU

There is growing recognition that the patient voice needs to be heard in the health technology assessment (HTA) process to ensure their needs and preferences are accurately reflected in access decisions. Patients are already involved to different degrees in the HTA processes in several European countries, for example Germany, France, and the UK, which helps to ensure the interests of those patients are reflected in reimbursement decision making.

Nevertheless, access equality and patient involvement remain a problem in many EU countries. This is illustrated in the EFPIA Patients W.A.I.T. Indicator 2022 Survey, which found a significant difference in the rate of full availability of medicines to patients in European countries, ranging from 99% in Germany to just 7% in Serbia. Additionally, the average time to reimbursement for innovative treatments across EU and European Economic Area (EEA) countries varies greatly from 133 days in Germany to 899 days in Romania.

JCA patient involvement

It is hoped that these patient access barriers will be addressed with the implementation of the Joint Clinical Assessment (JCA). As a key pillar of the EU's Health Technology Assessment Regulation (HTAR), the JCA seeks to centralize and harmonize the assessment of clinical evidence and thereby accelerate patient access to innovative medicines. It also adds both a new element to the access discussion, as well as an opportunity for industry and patient groups to advocate and educate on access.

The new HTA regulation provides for involvement of patients in JCA via the HTA Stakeholder Network that comprises 44 member organizations including patient organizations, non-governmental organizations in the field of health, health technology developers and health professionals.

The importance of integrating the patient voice is also enshrined in the draft of the first implementing act: *"The JCA subgroup should aim to select patients, clinical experts and other relevant experts who have the required expertise in the therapeutic area of the joint clinical assessment, from a European or international perspective. These experts should be consulted during the joint clinical assessment,"* the draft act states.

While the JCA will be jointly conducted at EU level, pricing and reimbursement decision-making will remain the responsibility of individual countries; however, regional HTA

bodies are obliged under the HTAR to 'give due consideration' to the JCA reports and to take into account input from stakeholders, including patients. As the authors of the HTx project report note: *"...there is still and will remain, a need for individual EU countries to conduct HTA with proper patient involvement."*

Patients' special role in rare disease assessments

The JCA process will be mandatory and will begin in January 2025 with advanced therapy medicinal products (ATMPs), which often target patients living with rare diseases, and oncology products, including their rare indications. Other orphan products will be subject to JCA as of January 2028.

Rare disease products pose additional challenges to HTAs due to the small patient populations, insufficient knowledge of the natural history and a lack of standard of care or any alternative therapies to name just a few. In this context, it is of even greater importance to ensure that patients are heard. They have unique lived experience, are often the expert of their own condition and are the ones that need to be consulted to define which outcomes are meaningful and matter to them in their daily lives.

To date, the acceptance of patient evidence in HTA/access decisions has been inconsistent. According to an Executive Insight report, 38% of payers say patient evidence is often (13%) or always (25%) considered in HTA/access decisions in their country, while 85% of patient experts believe patient evidence is not adequately considered in decision-making.

EFPIA (European Federation of Pharmaceutical Industries and Associations) and EURORDIS, the European umbrella group for rare disease patient organizations, in 2022 issued a joint statement with proposals aiming to better adapt HTA frameworks to the inherent challenges posed by orphan medicines.

In the context of the JCA, the Alliance for Regenerative Medicine (ARM) has also raised concerns about how the assessments of ATMPs will be impacted. For example, the complexity of demonstrating durability of effect with ATMPs presents a source of uncertainty, which is why accepting and harmonizing EU-wide collection of real-world evidence (RWE) in the JCA process is an imperative. Here patient organizations can and should play a central role, also with the support of pharmaceutical companies, to for example conduct patient preference studies, run patient surveys, and collect data on patient-relevant outcomes that should be considered as 'hard outcomes' and hence be integral to the JCA decision-making process.

Empowering patients for JCA

With the new JCA process explicitly including patient representation, it will be critical to ensure that patient groups or selected patient experts are literate in the process. There will also be a need for upfront clarity on what the expected patient input looks like, and transparency on how that input feeds into the decision-making process, including potential appeal mechanisms.

The European Union has co-funded an initiative that seeks to provide patients and patient advocacy groups (PAGs) with the knowledge they need to participate in the HTA process. EUCAPA trains patients and their representatives in the HTA decision-making process, providing them the knowledge and skills to advocate for access to innovative treatments for rare diseases. A consortium of patient groups, including EURORDIS and the European Patients Forum (EPF), are involved in the initiative and are focused on working with patients to build capacity and knowledge to advocate at a national or European level.

Industry can also play a role in strengthening the patient voice, generally by involving patients systematically and early on in the medicine lifecycle to help define, capture and co-create patient experience data and more patient-relevant endpoints in clinical trials, as well as in interpreting and disseminating patient outcomes for JCA and beyond.

What remains to be seen is what exactly the timing and interactions will be for involving patients and the patient perspective in the JCA process. Patient groups and patient experts will need support and enough lead time to prepare and be trained to effectively provide input in the JCA. Larger regional or umbrella patient organizations will likely be more proficient here already, but national PAGs and also smaller patient groups or individual patients, especially for (ultra-) rare diseases, will need to be supported to enable their effective participation.

Another key question is also how patient representatives will be selected for the JCA. Patient experts need to be able to both represent the views of the broader community and provide complex information, e.g. on patient preferences as mentioned above. All of this calls for strengthening the role of patient organizations to ensure legitimate and fair representation of the overall patient community's views in the JCA.

Looking into the future

There are many reasons to be hopeful that the patient voice will be given greater weight in the harmonized JCA process and the overall intentions of harmonizing, accelerating and broadening patient access are very good.

Strong patient representation in the JCA by early and continued involvement of patients that are properly trained and able to represent the community, could set the precedent and further push for inclusion of the patient voice in all national HTAs where final decisions on reimbursement will continue to be made.

The key measure of success for the JCA will be whether it will be able to increase transparency, reduce duplication and ultimately accelerate and improve access to innovative treatments for all patients irrespective of their condition.

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