Optimizing the involvement of EFCCA patients in medicines-related decision making

EFCCA
European Federation of Crohn's & Ulcerative Colitis Associations

Optimizing the involvement of EFCCA patients in medicines-related decision making

Project carried out by:

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European Federation of Crohn's & Ulcerative Colitis Associations

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[LIST OF ABBREVIATIONS]

ABBREVATION	DESCRIPTION
DIVA	Data Insights for added therapeutic Value
ECCO	European Crohn's and Colitis Organisation
EFCCA	European Federation of Crohn's and Ulcerative Colitis Associations
EFPIA	European Federation of Pharmaceutical Industries Associations
EMA	European Medicines Agency
EU	European Union
EUnetHTA	European Network for Health Technology Assessment
FDA	U.S. Food and Drug Administration
HTA	Health Technology Assessment
IBD	Inflammatory bowel disease
JCAs	Joint Clinical Assessments
NGOs	Non-governmental organizations
NICE	National Institute for Health and Care Excellence
PICO	Population, Intervention, Comparator, Outcomes
P&R	Pricing & Reimbursement
PRO	Patient-Reported Outcomes
PROMs	Patient-reported outcome measures
QoL	Quality of life
RCT	Randomized Clinical Trials
RWE	Real-World Evidence

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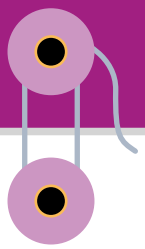

[CONTEXT AND JUSTIFICATION]

In recent years, the active participation of patients in the medicines decision-making process has become increasingly relevant, positioning us as key agents in a healthcare system that values our experiences and preferences to improve the quality of treatment and clinical outcomes.

The European Federation of Crohn's and Ulcerative Colitis Associations (EFCCA) is regularly involved in several key processes related to the health and well-being of patients living with inflammatory bowel diseases (IBD), such as Crohn's disease and ulcerative colitis. Some of the main processes in which we participate are the evaluation of medicines at European level, the promotion of disease research and the collaboration with national patients' associations in Europe defending their rights. EFCCA carries out these functions through several channels:

1. Official meetings:

EFCCA participates in official meetings when medicines-related issues that impact us are being discussed.

2. Email correspondence:

EFCCA engages in informal email exchanges to resolve queries and requests sent to us by the European Medicines Agency (EMA).

3. Consultation processes and discussion forums:

EFCCA contributes to consultation processes and forums concerning the new

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Health Technology Assessment (HTA) regulations.

4. Organization of education and awareness programs:

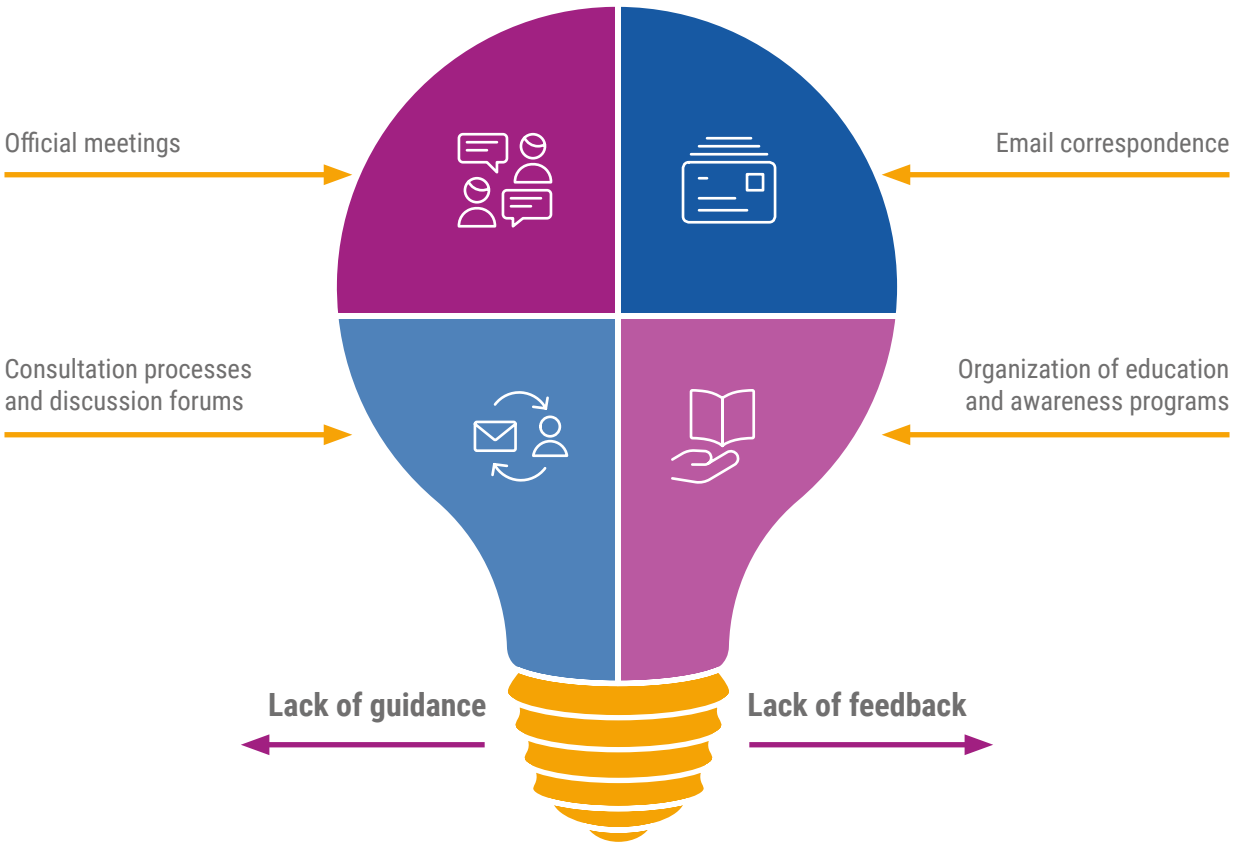
EFCCA organizes events, conferences and information campaigns at the European level to improve knowledge about these diseases and reduce the associated stigma.

The focus of these interactions predominantly turns around similar themes: the risk-benefit balance and comparative value from the patient's perspective, patient-reported outcomes, our experience with the disease and our views on current treatments and the attributes that new medications should possess to address the ongoing challenges faced by patients.

Recognizing the significance of our involvement, EFCCA is committed to providing relevant, accurate, and high-quality inputs that can help to solve doubts or guide decisions. Despite our active engagement, **EFCCA faces several challenges**, since guidance and feedback about our contributions is not consistently offered and framed.

Lack of guidance: we have not received explicit instructions regarding the specific concerns of evaluators and payers, nor their expectations from us, the topics they are most interested in, and the preferred methodology and format for our input.

Feedback on our contributions: there is a lack of feedback on the usefulness of our contributions so far, making it difficult to determine what to improve, add, or discard.



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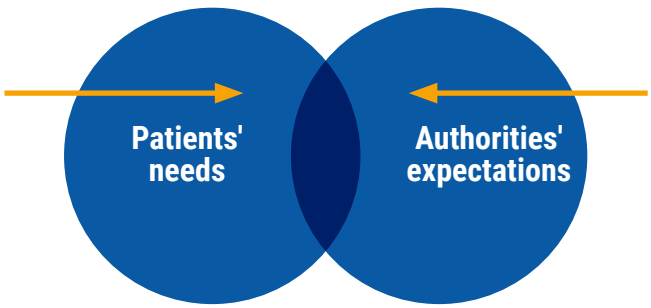
02

[OBJECTIVES]

This initiative aims to bridge the gap between the real needs of patients and the authorities expectations by developing a more structured and informed approach to EFCCA's contributions. Doing so, the impact of patient participation in the process of evaluating medicines and medical devices can be improved, promoting a more inclusive health technology assessment and enhancing innovation and research in IBD.

Therefore, the main aim of the initiative is to optimise the participation of patient associations in the HTA process and to ensure that our input is aligned with the expectations of European regulators. Many of the recommendations and best practices derived from this initiative are applicable at both the European and national level. Moreover, the benefits are not intended to apply only to EFCCA but also to other patients associations and to the system in general.

»»» Figure 1. Main objective of the initiative: bridging the gap



As is ... to be ... to do

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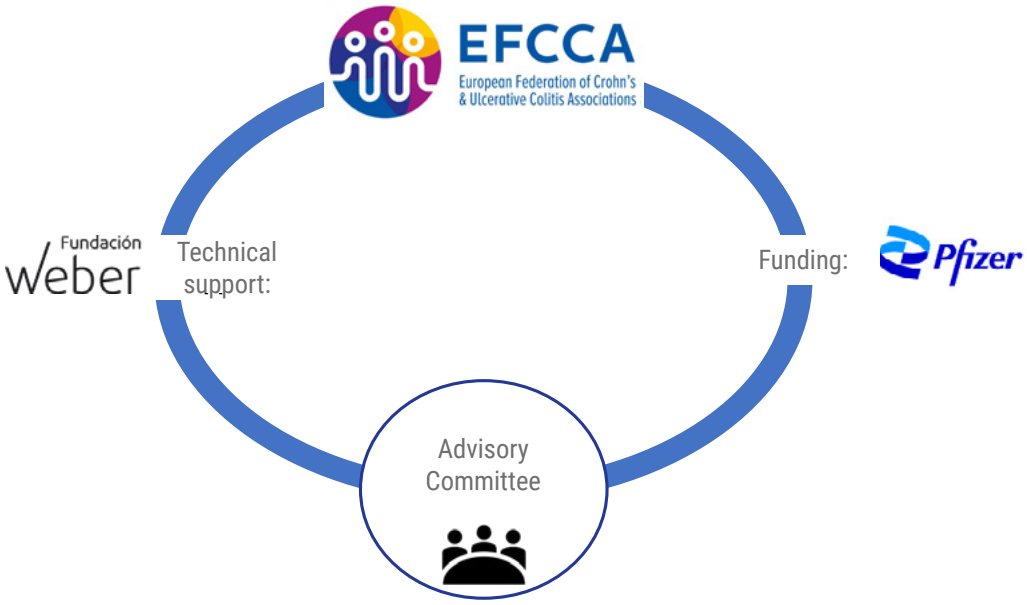
[WORKING METHODOLOGY]

This initiative was done through an Advisory Committee, set up to get their point of view and advice on the current situation. In addition to this, a literature review was performed using Pubmed and Google Scholar as main sources.

The initiative was carried out by the EFCCA, with the technical support of the Weber Foundation, which has provided its experience and knowledge in the implementation of patient-centered health technology assessment processes. In addition, it has had the unconditional

»»» Figure 2. Initiative participants



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financial support of Pfizer, which has made it possible to secure the necessary resources to develop and carry out this important work. The initiative was focused on the discussion and feedback of a committee composed by international experts.

The Advisory Committee was multidisciplinary and consisted of six experts from various fields, including health decision-making, HTA bodies, and academia, as well as a clinician representative of the European Chron's and Colitis Organisation (ECCO) (»»» Table 1).

The functions of the Committee's experts included the following tasks:

- **Pre-work of the meeting.** The preparation of the meeting was key to achieving the proposed objectives. Experts were invited to answer a set of questions included at the pre-reading document prepared by Weber, so that the meeting could be conducted in a more effective and productive manner.

- **Committee meeting.** The meeting was the core of the work, allowing us to analyze the situation, share experiences, and propose

»»» Table 1. Composition of the Advisory Committee

	Name	Position, country
Meeting No1 (June 2024)	Chantal Bélorgey	Ex Head of HTA at French National Authority for Health (HAS), France
	Meindert Boysen	Ex Head of International Affairs at the National Institute for Health and Care Excellence (NICE), England
	Luka Voncina	Expert in HTA processes, Croatia
Meeting No2 (September 2024)	Claudio Jommi	Full professor of Management, Department of Pharmaceutical Sciences, Università del Piemonte Orientale, Novara, Italy
	Jorge Mestre	Expert in pharmaceutical policy, Spain. Profesor Asociado, Universidad Carlos III, Spain
	Alessando Armuzzi	Inflammatory Bowel Diasease Member of the Center. IRCCS Humanitas Research Hospital. Humanitas University. Milan, Italy. European Chron´s and Colitis Organisation (ECCO)

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actions for improvement. Due to scheduling problems, the committee was finally divided into two different online meetings (with three experts in each of them). The first one was held on 24th June, and the second on 19th September 2024.

- **Post-meeting.** The experts were invited to review and validate the documents resulting from the meeting, which included the minutes of the meeting and this report of results. Both drafts were prepared by Weber.

During the two meetings, 13 key questions were addressed ranging from understanding patients' needs to how to better integrate our voice into regulatory and pricing decisions. The experts attending the meetings contributed to the development of the initiative through discussion and debate aimed at addressing the key issues raised at the meetings. At the same time, the experts put forward some recommendations on what EFCCA and other stakeholders should do to improve our participation in the process.

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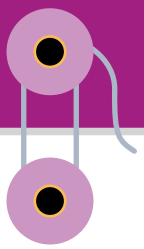

[SUMMARY OF THE CURRENT SITUATION]

The current landscape of health technology assessment is undergoing a significant transformation, driven by regulatory changes and an increasing demand and supply of more effective, patient-centered healthcare solutions. These regulatory reforms seek not only to enhance the evaluation and approval processes for new technologies, but also to ensure that the treatments and devices introduced into the market are safe, effective and accessible to the population.

4.1 Health technology assessment

The European Medicines Agency is responsible for the scientific evaluation of medicines and provides recommendations on marketing authorizations. However, the actual granting of marketing authorization is performed by the European Commission[1]. Once a product has been approved by the EMA, based on its benefit-risk assessment, it tipically undergoes national HTA to assess its added value and broader societal impact, ensuring that medicines are not only effective but also provide good value for money and benefit the population as a whole.

HTA is a multidisciplinary process that uses explicit and scientifically robust methods for assessing the added value, effectiveness, costs and broader impact of health care interventions. The process of HTA involves

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multiple stages and entities, including regulatory authorities like HTA agencies, and national health systems. After HTA evaluations, national health authorities decide whether to reimburse the technology and under what conditions[2].

HTA has emerged as an essential tool for demonstrating comparative effectiveness and value of new technologies, prioritizing those that offer better health outcomes at a reasonable cost. Overall, HTA encompasses a broad range of evaluative aspects. On the one hand, it includes clinical domains that require assessment, such as relative safety, clinical effectiveness, and patient reported outcomes. On the other hand, HTA also covers non-clinical domains, which encompass economic, social, ethical, legal or organisational aspects. These non-clinical considerations are subject to national analysis and are evaluated by individual Member States to ensure that health technologies align with local healthcare priorities and regulations[3] (Figure 3).

4.2 The new European HTA regulation

In recent years, significant changes in HTA regulation have emerged to adapt to the evolving healthcare landscape. These modifications aim to reduce differences in access across member states, and to enhance the efficiency and transparency of assessments at the European level, while enhancing the participation of the different stakeholders.

Until 2021, HTA in Europe was based on voluntary cooperation, through the HTA Network and the EUnetHTA joint actions. The HTA Network connected the national authorities in charge of HTA, in compliance with Directive 2011/24/EU. This collaboration produced strategic documents that were subsequently used in the joint EUnetHTA actions. These actions, funded through EU health programs, were carried out in three phases: developing joint clinical activities related to HTA (2010-2012); testing the methodology and

»»» Figure 3. Clinical domains vs non-clinical domains

CLINICAL DOMAINS	• Health problems and currently used health technologies (e.g. medicines, medical devices, surgical procedures). • Description of Health Technology under assessment • Relative clinical effectiveness. • Relative safety.
NON-CLINICAL DOMAINS	• Economic evaluation. • Ethical aspects. • Organisational aspects. • Social aspects. • Legal aspects.

Source: European Commission (2023)[3].

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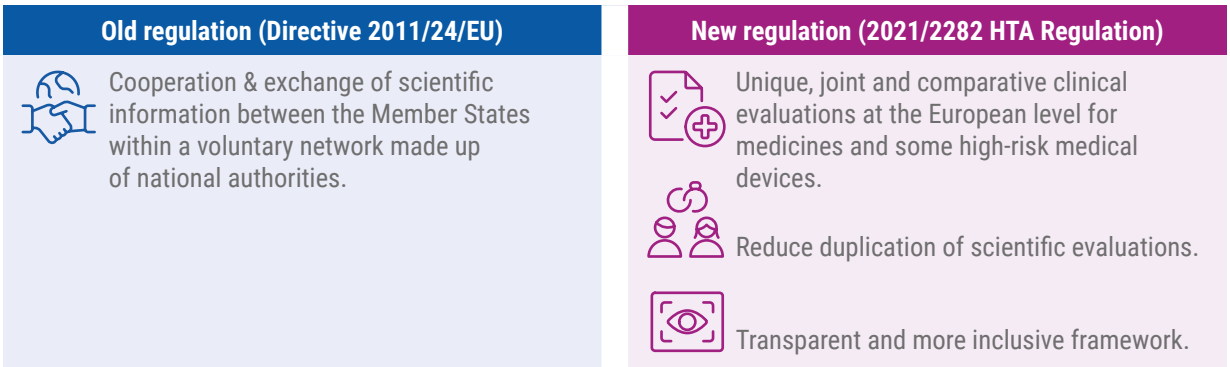
activities developed during the first joint action (2012-2015); and producing joint scientific reports (2017-2021)[4].

In December 2021, the new HTA Regulation (Regulation 2021/2282 of the European Parliament and of the Council of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU) was approved to provide a framework for cooperation between the Member States on health technologies at the European Union[5]. The new HTA regulation represents a significant advancement in cooperation among Member States by incorporating joint clinical assessments. This approach aims to optimize resource utilization, enhance effectiveness by preventing the duplication of scientific evaluations, and establish a more transparent and inclusive framework for patient participation. In contrast to the previous regulation, the new regulation promotes a collaborative and standardized assessment process (»»» Figure 4)[5,6].

The new regulatory framework introduces substantial changes, highlighting the mandatory implementation of Joint Clinical Assessments (JCAs) for medicines and medical devices of higher risk classes, as well as for in vitro diagnostic medical devices and other health technologies. These assessments should be taken in due consideration at the national level, complemented by evaluations of non-clinical domains. In addition, it includes Joint Scientific Consultations (JSC) and a horizon scanning process that allows for the identification of emerging health technologies. The latter process is critical, as it enables the detection of trends and challenges in science and technology that are relevant to medical research and development, ensuring that the health system is prepared to integrate innovations that can improve patient care and outcomes[5]. In addition to these advancements, the new framework emphasizes the importance of standardized methodologies for conducting JCAs and JSCs[3].

»»» Figure 4. Main differences between the old and the new European regulation



Sources: Regulation 2021/2282/EU[5], Directive 2011/24/EU[6].

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For the implementation of the new European HTA Regulation, the Coordination Group plays an essential role in coordinating JCAs among the Member States, setting priorities for these assessments, facilitating the exchange of relevant information, promoting the consultation and participation of experts and stakeholders, providing technical and methodological support, and preparing reports and recommendations (»»» Figure 5)[5].

To ensure inclusiveness and transparency in working together, the Coordination Group will consult with a broad spectrum of stakeholders, including patient organizations, health professionals, clinical and academic societies, health technology developers, consumers and other non-governmental health organizations. A stakeholder network is established to facilitate dialogue with the Coordination Group.

These experts are selected for their specialized knowledge in the field and will operate independently while upholding confidentiality and avoiding financial interests

»»» Figure 5. Responsibilities of the Coordination Group under the new HTA Regulation

Facilitation of Joint Clinical Assessments (JCAs)	Support for Joint Scientific Consultations (JSCs)	Horizon Scanning	Standardization of Methodologies
The Coordination Group oversees the execution of JCAs, ensuring that assessments of medicines, high risk medical devices, and in vitro diagnostic devices are conducted collaboratively and consistently among Member States.	The Group coordinates JSCs, creating a forum for stakeholders to engage in discussions regarding the scientific and technical aspects of health technologies prior to their market introduction.	The Coordination Group is tasked with implementing horizon scanning activities, which involve monitoring and identifying emerging health technologies and trends that may influence healthcare systems.	The Group promotes the adoption of standardized methodologies for JCAs and JSCs, ensuring that evaluations are based on robust evidence and consistent criteria across Member States.
Stakeholder Engagement	**Compliance Monitoring**	**Capacity Building**	**Reporting and Transparency**
The Coordination Group facilitates stakeholder participation in the assessment process, ensuring that diverse perspectives—particularly those of patients and healthcare providers—are incorporated into evaluations and decision-making.	The Coordination Group monitors adherence to the new HTA regulation among Member States, ensuring that established processes and standards are followed and that best practices are disseminated.	The Coordination Group may engage in initiatives to build capacity, providing guidance and resources to Member States to strengthen their HTA capabilities and foster a collaborative culture.	The Group is responsible for ensuring transparency in the assessment process by reporting findings, methodologies, and recommendations to relevant stakeholders, thereby fostering trust and accountability in the HTA system.

Source: adapted from Regulation 2021/2282/EU[5].

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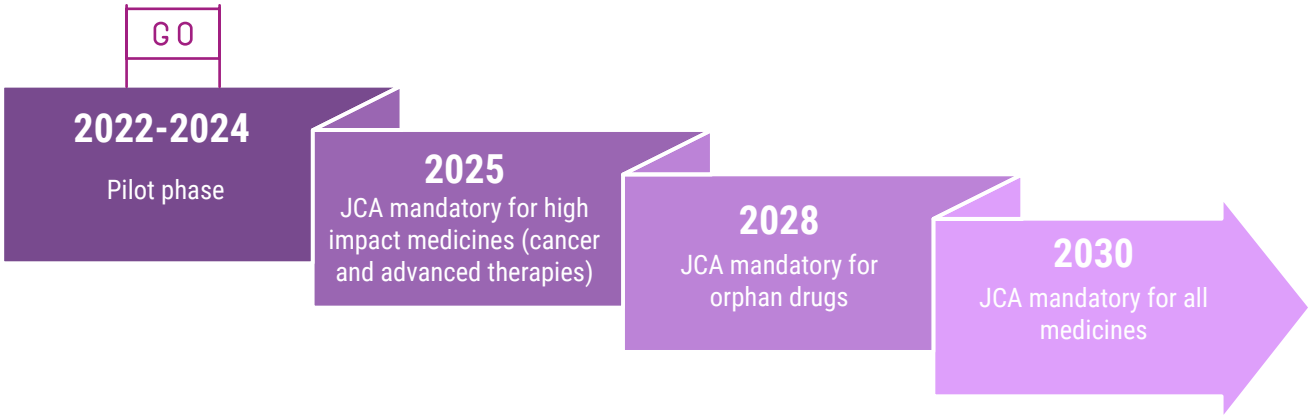
or other conflicts of interest. The Coordination Group will meet at least once a year with the stakeholder network to provide updates on progress and promote information exchange. In addition, patients and other experts will have the opportunity to contribute to the development of the final document of the joint scientific consultation, through face-to-face or virtual meetings, allowing for the sharing of perspectives.

Concerning the timetable for the implementation of the HTA Regulation, the process will start with a limited number of evaluations, which will progressively expand over time. Following an initial pilot phase, the implementation of Joint Clinical Assessments for oncological medicinal products and advanced therapies is scheduled to begin in 2025. Starting in 2028, the assessments will also include medicines for rare diseases. By 2030, the regulation will extend to encompass all other medicines and higher-risk medical devices, as well as those medical devices and in vitro products selected by the Commission based on recommendations from the Coordination Group (»»» Figure 6)[5].

In addition, the new regulation establishes detailed rules to harmonize methodological standards and encourage cooperation and interaction between the various stakeholders, including patients. The harmonization of criteria at the European level and the promotion of collaborative approaches between the different assessment bodies are central aspects of this new regulation, with the aim of facilitating the adoption of health innovations and improving patient outcomes.

»»» Figure 6. Schedule for the implementation of JCAs



Note: for high risk Medical Devices, JSC are supposed to start in 2025 and JCA in 2026 or 2027. Medical devices classified in classes IIb or III pursuant to Article 51 of Regulation (EU) 2017/745 and in vitro diagnostic medical devices classified in class D pursuant to Article 47 of Regulation (EU) 2017/746, as well as for medical devices for which the relevant expert panels referred to in Article 106.1 of Regulation (EU) 2017/745 have delivered their opinions or opinions.

Source: adapted from Regulation 2021/2282/EU[5].

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4.3 Benefits of the new HTA regulation

The new HTA Regulation represents a significant advance in the way health technologies are assessed and implemented in Europe, and offers a number of crucial benefits for patients and other stakeholders. It establishes a more consistent and transparent framework for the evaluation of medicines and medical devices, promoting a better use of HTA capacity and greater collaboration between Member States. It aimes to present the evidence and science in a consistent manner to local decision makers, although it is important to note that JCA is only the start of an evaluation at llocal level. HTA should promote the consideration of patient perspective in those countries where it is disregarded. Therefore, thanks to this new regulation, patients can benefit from potentially faster and more equitable access to innovative treatments that have proven efficacy and safety, as well as better alignment of evaluations with their needs and expectations. In addition, by encouraging the inclusion of the patient perspective in assessments, the person-centered approach to healthcare is strengthened, contributing to improved quality of life and health outcomes for those living with chronic or complex diseases[7].

For decision-makers, it provides a clearer and more harmonized framework for evaluating the effectiveness and safety of new technologies, enabling more informed and evidence-based decisions. It establishes a more consistent and transparent framework for the evaluation of medicines and medical devices, promoting

»»» Figure 7. Expected benefits from the new EU HTA Regulation

Decision-makers	Patients	Industry
• Timely and high quality scientific reports. • Ability to improve sustainability. • Proven added-value to support decisions inspired by value-for money. • Knowledge sharing. • Avoid duplication of efforts. • Better use of human and financial resources.	• Potential for greater speed and alignment. • Higher level of protection. • Greater transparency and engagement in the HTA process. • Faster assimilation of promising innovative technologies.	• Unique, joint and comparative clinical evaluations at the European level for drugs and some high-risk medical devices. • Reduce duplication of scientific evaluations. • Transparent and more inclusive framework. • More consistent and predictable assessment process.

Source: prepared by the authors based on Regulation 2021/2282/EU[5] and Giorgio (2021)[8].

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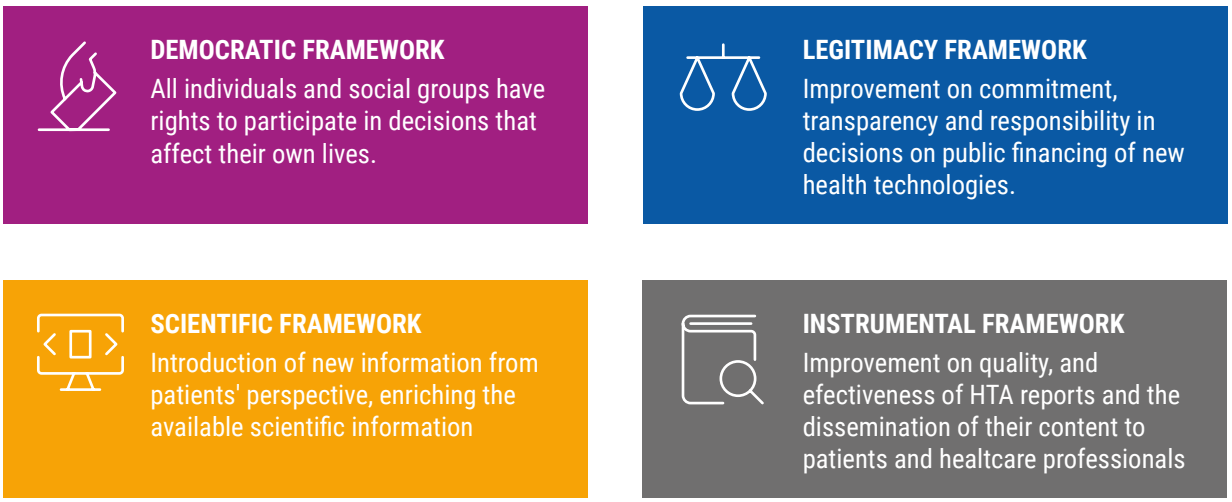
a better use of HTA capacity and greater collaboration between Member States. It aimes to present the evidence and science in a consistent manner to local decision makers, although it is important to note that JCA is only the start of an evaluation at local level (»»» Figure 7)[5,8].

The participation of patients in HTA is justified by several key values. Firstly, it upholds democratic principles by ensuring that the voices of those directly affected by health technologies are heard, and taken into consideration in the appraisal process.

This inclusion also boosts the legitimacy of the HTA process, making assessments more relevant and trustworthy by reflecting the real-world experiences of healthcare users. Additionally, patient engagement may enrich HTA by providing unique insights into treatment preferences and the impacts of health technologies on quality of life. Finally, considering patient perspectives can lead to more effective health technology assessments, resulting in the selection of technologies that better meet patient needs and ultimately improve health outcomes (»»» Figure 8)[9].

»»» Figure 8. Values that justify the participation of patients in HTA



Source: Toledo-Chávarri (2019)[9].

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05

[RECOMMENDATIONS]

In this section, the reader will find a detailed analysis of the points raised in the expert meetings. We will begin by exploring the key topics discussed, ranging from the need for greater inclusion of the patient voice in the assessment process to the importance of establishing clear and harmonized criteria at the European level. Then, we will link the identified challenges for patients in relation to access and participation in health technology assessment with a series of practical recommendations in different areas. Through this comprehensive and structured approach, we seek to provide a clear framework for improving the evaluation and, ultimately, the care of patients with IBD.

5.1 Key topics dicussed

In our efforts to improve the understanding and treatment of IBD, EFCCA works closely with our member associations to develop information and support resources. As part of this initiative, the federation has compiled a set of generic questions which can serve as a valuable starting point for assessing our needs and the expectations of health care evaluators and payers. These efforts not only seek to facilitate health technology assessment, but also to ensure that our voice is heard and integrated into the decision-making process. Many of the questions that we have had to answer in recent years are compiled at the EUnetHTA 21 template (»»» Figure 9)[10].

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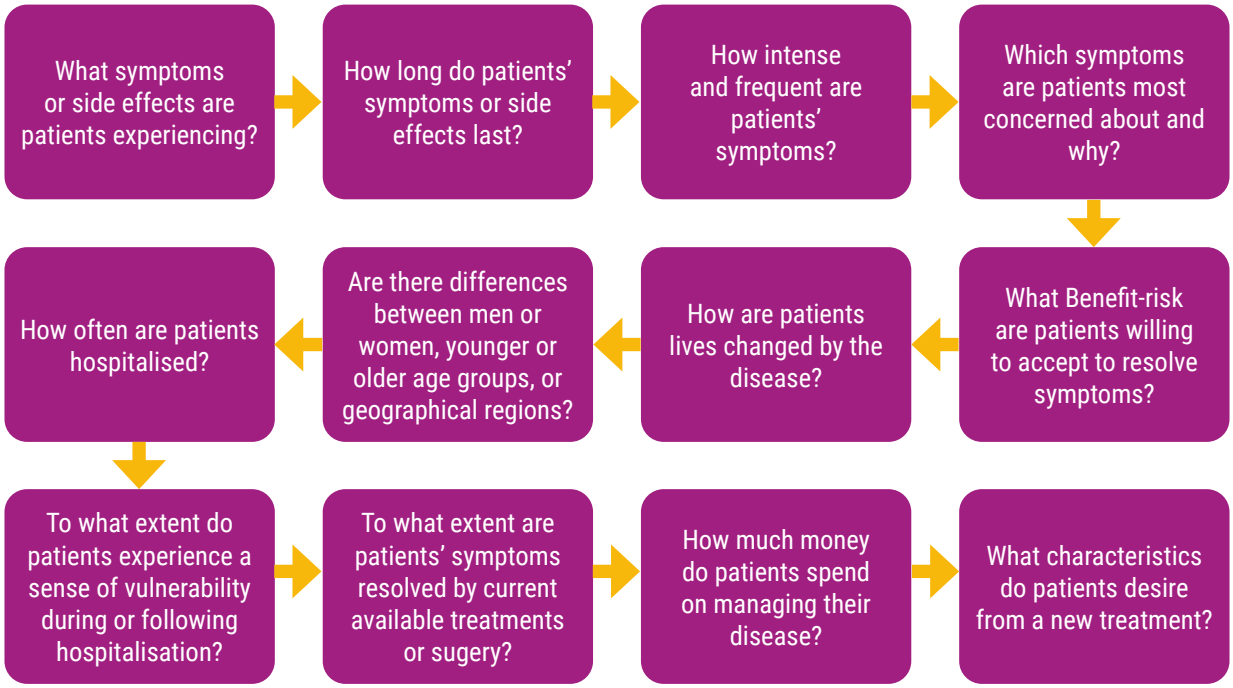
»»» Figure 9. Questions that EFCCA representatives have had to answer in recent years



Source: EUnetHTA (2023)[10].

The experts on this initiative agreed that the questions posed are appropriate, although they also emphasized other critical aspects that are not often included at clinical trials and conventional assessments. Ultimately, the main aim should be to understand how treatments truly impact patients' lives, expectations, and unmet needs, some aspects often overlooked in traditional methodologies.

On the one hand, experts pointed out to the importance of having a clear and objective measurement on the impact of the diseases on health-related quality of life (HRQL). It would be important to develop and apply generic and specific HRQL scales. In the case of IBD, the most common symptoms considered are diarrhoea, bleeding and abdominal pain. However, they may vary according to disease severity and phenotype. Also, other issues such as fatigue should be considered.

On the other hand, experts highlighted that there are other relevant topics that it would also be interesting to consider in the assessment, appraisal and decision-making process, such as patient convenience, avoided hospitalizations or surgical interventions, the impact on labour productivity or new PRO that better detect heatlh-realted quality of life (»»» Figure 10).

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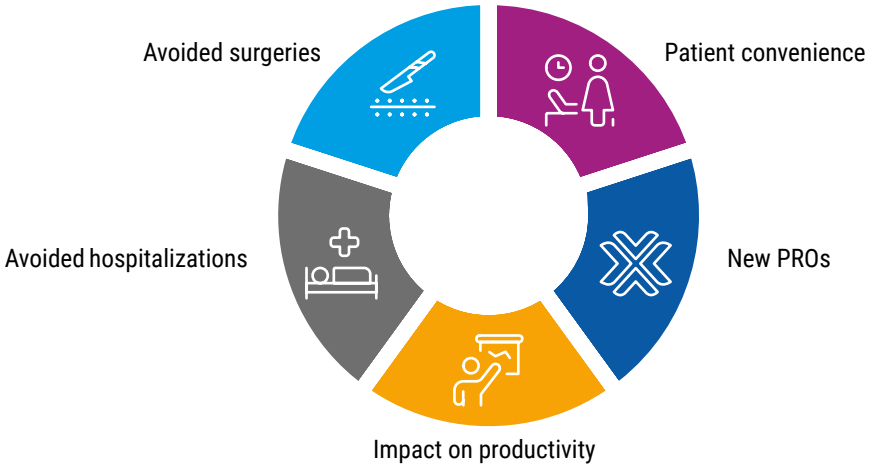
»»» Figure 10. Relevant additional topics to address



Note: PROs: patient reported outcomes **Source:** own preparation.

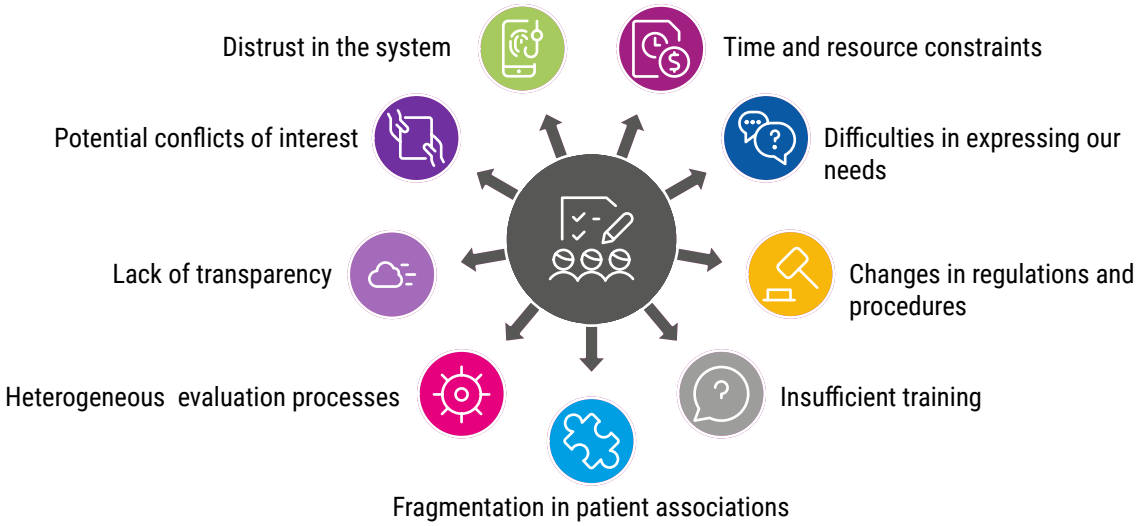
5.2 Recommendations for the challenges identified

In trying to contribute to the evaluation of health interventions, patients face a variety of challenges (»»» Figure 11), which were the focus of the recommendations of the experts of this committee.

5.2.1 Data to justify patients´ answers

Along the healthcare decision-making process, the collection of evidence is crucial to show the impact of the disease or the relative impact of the assessed drug. However, the type of evidence used may vary according to the availability, quality and applicability of the data.

»»» Figure 11. Main challenges for patient involvement in HTA



Source: own preparation.

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There are two main types of evience: scientific and patient-reported evidence. Scientific evidence is based on objective data obtained through clinical trials, experiments and statistical analysis. It is considered the most robust and reliable, since it is based on standard, controlled and verifiable methodologies, such as randomized controlled trials or observational studies. Patient-reported evidence, on the other hand, comes from patients' experiences and opinions. Although subjective, they can also be scientific-based through specific analyses that can make more generalizable the expressed experience, and offer valuable insights when scientific evidence is limited or difficult to obtain. It can reveal important qualitative aspects, such as improvement in quality of life or personal preferences about treatments. This information is usually obtained through surveys, focus groups or interviews, among others (»»» Figure 12).

Overall, scientific evidence is preferred by regulators and HTA agencies because of its objectivity and accuracy. However, it can be costly and time-consuming to collect, especially in regions with less research capacity. In contrast, testimonial evidence provides a direct perspective from patients, which makes it also a useful tool for understanding their needs and expectations. However, testimonial evidence is more subjective and poses other challenges in terms of comparisons, generalisation and establishing causality. Therefore, it is less commonly accepted in regulatory decisions without complementary scientific support. All experts agreed that every decision should be taken based on the best possible evidence at that moment.

Decision-making based solely on scientific data may not always capture the full picture. In this sense, combining scientific evidence with testimonial evidence would be an effective strategy for gaining a more holistic view of healthcare treatments and techonologies. It is essential for every bit of evidence to be linked to specific questions to be addressed in the HTA process. Additional evidence on different population subgroups, with varying needs, may reflect real-life diversity and priorities.

»»» Figure 12. Scientific evidence versus testimonial data

Scientific evidence	Testimonial data/ Patient-reported evidence
It is based on objective data obtained through clinical studies, experiments and statistical analysis. • Provide clear facts. • Provide information of experiences to offer an accurate and balanced overview of patients' and caregivers perspectives.	**It comes from patient's experiences and opinions.** • Measure the impact of the disease/treatment on daily life • Patient surveys • Focus groups • Interviews

Source: own preparation.

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»»» Figure 13. Recommendations on evidence generation

Main issues	Recommendations
• Need to provide data to justify our answers. • Resource and time constrains to generate data. • Testimonial data are usually not considered in the decisions. • Different criteria among countries for determining the relevance of scientific evidence for decision making.	• Initiate discussions with industry early in the lifecycle of the health technology. • Develop our own studies to generate data. • Early involvement of patients in the development of healthcare technologies. • Harmonize data collection criteria at the European level.

Source: own preparation.

This mix seeks to balance the objectivity of quantitative research with the subjectivity of personal experiences. For this reason, patient organizations should try to develop independent studies/surveys to generate and collect data, adopting the most rigorous and robust approach in the study design. Experts recommended patients to prioritize impactful contributions and to be concise, emphasizing that the key messages should not exceed five statements. Additionally, it would be of interest to initiate discussions with industry early in the lifecycle of health technology (e.g. in JCSs) to define PROs that truly reflect patient needs, and ensure that they are finally included in trials (»»» Figure 13).

5.2.2 More training needed to understand and generate scientific evidence

Patient understanding of HTA is critical. Patients may need to review HTA technology developer documentation and provide input on protocol advice, outcome selection, study design, and comparator choice, to advocate for outcomes that matter most to patients. However, HTA processes and methodologies, including indirect comparisons and protocol development, can be complex, especially for those without scientific training.

Moreover, generating scientific evidence involves understanding research methodologies, statistical analyses, and regulatory requirements, areas in which patients may not be trained. This knowledge gap, together with the lack of adequate resources, can hinder effective patient involvement in HTA. Therefore, establishing training programs aimed specifically at patient representatives could help bridge this knowledge gap.

To effectively engage patients in HTA processes and contribute to generate scientific evidence, patients and patient advocates should familiarize themselves with HTA reports and methodologies, understanding the terminology, structure,

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»»» Figure 14. Recommendations on training

Main issues	Recommendations
• Complex and changing HTA methodologies and processes that could be difficult to understand. • Lack of adequate information about available treatments and resources. • Difficulty in transforming needs and preferences in a formal way.	• Learn how to review HTA documentation. • Learn how to provide input on protocol advice, outcome selection, study design, and comparator choice. • Comprehensive training in HTA methodologies, including courses on statistical methodologies, to understand indirect comparisons. • Continue working on highly specific strategies tailored to each condition. • Plan in advance. • Use available online resources and training programs.

Source: own preparation.

and scope of these assessments. It would be advisable to receive comprehensive HTA methodology training (statistical approaches like indirect comparisons and meta-analysis) to critically understand study results. To try to provide valuable insights, we should also work on tailored, condition-specific strategies, to develop specific expertise and offer more targeted feedback in HTA reviews.

To provide useful guidance and accessible options to enhance HTA knowledge, it is important to leverage available online resources and training programs (e.g. resources from EUnetHTA and programs from EFCCA) at both European and national levels. Collaboration with entities like HTA agencies, academia, scientific societies, pharmaceutical companies and other patient associations is also essential for strengthening patients' knowledge and training.

Finally, effective patient engagement also requires proper planning of training, collaborations, and foresight on upcoming products under evaluation. Through structured, forward planning, patient involvement becomes more systematic and impactful, helping HTA processes to better align with patients' actual experiences and priorities.

Patients need training to understand complex HTA methods and contribute effectively. Learning the basics, participating in training programs, collaborating with experts, and planning ahead for upcoming evaluations are essential.

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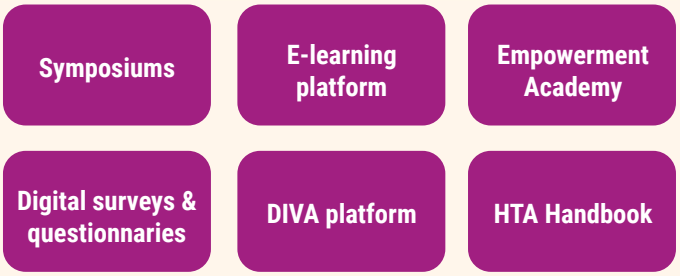
RESOURCES PROVIDED BY EFCCA TO MEET THESE CHALLENGES

EFCCA uses a variety of resources, innovative tools and developments in the HTA process to support patients with IBD (»»» Figure 15).

»»» Figure 15. Resources provided by EFCCA to face patient´s challenges



Source: adapted from EFCCA (2024)[11-14] and EFCCA (2018)[15].

Firstly, we promote the use of PROs, which are data directly reported by patients on their health status, quality of life and treatments. This allows for better assessment of the impact of treatments from the patient's perspective. We also conduct preference digital surveys or questionnaires to determine which treatment features patients value most. Specifically, in collaboration with the University of Leuven, we have conducted qualitative studies and surveys to identify patients' unmet needs and the most important features of treatments[13,14].

In addition to this, we are cooperating with organizations like ECCO and UEG developing digital tools and platforms. One of our newest tools is the DIVA (Data Insights for added therapeutic Value) platform, a digital tool designed to gather and disseminate data and information on IBD and approved drugs and clinical trials[12]. Additionally, Chat GPT has been included in the Charter IBD platform[16]. This tool offers access to personalized information and assistance in real time, facilitating informed decision-making and improving communication about IBD-related treatments and care.

We also offer an e-learning platform and the EFCCA Empowerment Academy, focused on empowering IBD patients through education and advocacy, providing them with the skills necessary to become trainers and advocates within their communities[15].

Lastly, we has recently published a handbook aimed at patients with IBD with the aim of explaining the drug approval and HTA process[11]. Highlights include the importance of active patient participation in these processes, and the need to understand patients' preferences and unmet needs. The guide highlights the crucial role of patients in collaborating with regulators to improve the development of therapies and ensure equitable access.

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5.2.3 Fragmented framework and engagement

The existence of several associations dedicated to the same disease presents significant challenges within the patient advocacy landscape. One of the primary issues is the fragmentation of efforts; multiple organizations working towards similar goals can dilute the overall effectiveness of advocacy initiatives. Instead of concentrating resources and attention on unified objectives, these associations often operate independently. The varying capabilities and uncoordinated nature of activities among these associations further exacerbates the problem, as they may encounter differing viewpoints and recommendations.

Moreover, patient feedback in healthcare assessments often lacks coordination, with input gathered inconsistently across a medicine's lifecycle. To increase the impact of patient perspectives, associations should have more regulatory influence, especially in reporting quality of life (QoL) metrics such as work productivity and specific PROs (e.g. fatigue in Crohn's disease). In JCAs, although patient data is structured, decision-making remains inconsistent across EU countries due to varying national approaches. Standardizing the information format through EU-wide guidelines and providing translations for accessibility would promote a more equitable and cohesive integration of patient input across Member States, ensuring that healthcare decisions reflect patient needs consistently.

In a fragmented advocacy landscape, establishing a unified patient voice on critical issues is crucial. Collaboration and coordination among patient associations will create a collective pool of resources and expertise, thereby enhancing the potential for impactful joint initiatives that benefit the entire patient community. Also, outside the clinical domain, which includes HRQoL, the HTA reglament encourages voluntary collaboration among Member States, a process that could be actively promoted by patient associations. Experts emphasized

»»» Figure 16. Recommendations to avoid fragmentation

Main issues	Recommendations
• Several small associations for the same disease. • Different capabilities. • Uncoordinated efforts.	• Avoid a fragmented representativeness: umbrella´s association. • Make a more structured interaction with other patients. • Give some framework or guidelines on your collaboration. • Translations for non-English speaker patients.

Source: own preparation.

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the importance of operating under a shared "umbrella" organization, enabling diverse voices to align and amplify their influence within HTA processes. This coordinated approach would help ensure that payers and HTA authorities receive a cohesive representation of patient perspectives on common concerns. Notably, initiatives such as the European Capacity Building for Patients (EUCAPA) project, launched by EURORDIS-Rare Diseases Europe under the EU4HEALTH initiative, exemplify promising steps toward building such coordinated capacity across patient organizations in Europe[17,18].

5.2.4 Heterogeneity across countries

Another significant challenge is the heterogeneity across countries, that stems from differences in healthcare infrastructure, reporting standards, and resource allocation, leading to uneven access to medicines, but also to data on treatment outcomes and decisions. As a result, patients in some countries may have access to comprehensive information that can inform advocacy, while those in other regions may face gaps in critical data. This variability complicates patient engagement and weakens the potential impact of patient voices in HTA processes.

Another barrier lies in the heterogeneity of data collection practices, comparator choices, and decision-making criteria across Member States. Each country adopts its own approach to gathering evidence, selecting treatment comparators, and applying specific criteria for HTA decisions. Consequently, patient perspectives, such as PROs on fatigue, administration mode or work productivity, may be emphasized in some contexts but neglected in others. This fragmented approach prevents the development of a cohesive, EU-wide strategy for patient involvement in HTA, as each country's priorities and methods may differ.

This heterogeneity contributes to inequities in patient access to treatments across the EU. Also, HTA organisations and payers are less sensitive to patient contributions when the budget costraints are more stringent.

»»» Figure 17. Recommendations to homogenize processes across countries

Main issues	Recommendations
• Available information vary across countries. • Heterogeneous data collection, comparators used and crteria applied in decisions. • Inequity in access to treatments. • Duplication of efforts and resources.	• Advocate for standarization in HTA processes. • Advocate for equal access to treatments. • Encourage collaboration between countries • Stay informed on HTA decisions and databases. • Organize information on your webpages.

Source: own preparation.

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The divergent HTA outcomes mean that while patients in one country may gain access to innovative treatments, those in another might not, even if they suffer from the same condition. Such discrepancies create a landscape in which access to healthcare is based more on location than on medical need. This inequity forces patient associations to advocate separately in each country, significantly complicating their efforts and undermining the goal of equal access to healthcare for all EU citizens. Furthermore, the lack of standardization in HTA processes leads to duplicated efforts and wasted resources across the EU. This redundancy not only increases costs but also burdens patient associations by requiring them to participate in parallel processes. With a more centralized or coordinated system, these resources could be better utilized, maximizing the quality and effectiveness of patient involvement.

Addressing these barriers requires a comprehensive EU-wide framework for HTA, focused on aligning processes and decision criteria across Member States. Standardizing data collection practices, treatment comparators, and decision-making structures, while ensuring that patients across all countries have access to the same information, would strengthen patient involvement and make HTA more responsive to patient needs.

Therefore, experts emphasized the importance of accurately capturing and understanding national healthcare contexts to create a process that is both inclusive and representative of the diverse characteristics and needs of individual countries' health systems. To promote greater alignment and initial standardization, experts recommended beginning joint evaluations with widely prevalent diseases, such as cardiovascular conditions or ulcerative colitis, and gradually expanding the scope of these assessments to include additional diseases over time across all EU countries.

For patient associations, awareness of information available on various HTA bodies' websites is essential. This requires maintaining familiarity with databases and decision records across all Member States, ensuring they stay informed on pertinent developments. For example,

Patients should stay informed about HTA processes, use online resources to track decisions, and advocate for standardized EU-wide frameworks to ensure equitable access to treatments. Engaging with experts and sharing relevant data can help amplify their impact.

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the EFPIA's Patients W.A.I.T. (Waiting to Access Innovative Therapies) inindicator,, which utilizes IQVIA data developed by the European Federation of Pharmaceutical Industries and Associations, offers a metric for tracking reimbursement timelines and access to technologies[19]. National databases on medicines may be also of interest. Additionally, patient associations could enhance accessibility by dedicating a specific section on their websites to collect relevant data on particular treatments, thereby simplifying access to information and contributing to patient education and engagement in HTA processes.

5.2.5 Wrong time, wrong reasons for involvement

Despite increased recognition of the importance of patient involvement, the current processes often lack the structure to include patient voices meaningfully and at the right stages. Experts have pointed out several current systemic issues that tend to involve patients too late and often only as a response to challenges of pharmaceutical companies. Late-stage involvement turns patient engagement into a reactive measure to resolve price and reimbursement disputes rather than a proactive approach to meet patient needs. Moreover, when patients are only approached after obstacles arise, it can create an impression that their insights are being used strategically rather than valuably, undermining trust in the process. The lack of a clear structure for their involvement and the influence of the pharmaceutical industry on decisions was also mentioned as an obstacle.

Ultimately, it is essential to avoid using patient involvement merely as a token gesture. Instead, the focus should be on ensuring that patient input is genuinely valuable in the assessment and impactful in the appraisal processes. Besides, early involvement would allow patients to highlight gaps in current treatment options, enabling HTA to assess the new therapy's true value

»»» Figure 18. Recommendations to enhance patient´s involvement

Main issues	Recommendations
• Lack of a clear structure for patient involvement. • Industry involving patients just if they receive a negative P&R decision. • Involving patients just to inform them, without listening to them. • Distrust in the process.	• Ask for a more structured interaction with regulators HTA bodies and industry. • Ask them to plan in advance and not to use patients at the end of the process or just as a box-ticking exercise. • Define in which areas patient associations can be useful, distinguishing technical from political issues. • Prioritize impactful contributions that distinctly influence HTA and decision-making processes, ensuring clarity and effectiveness.

Source: own preparation.

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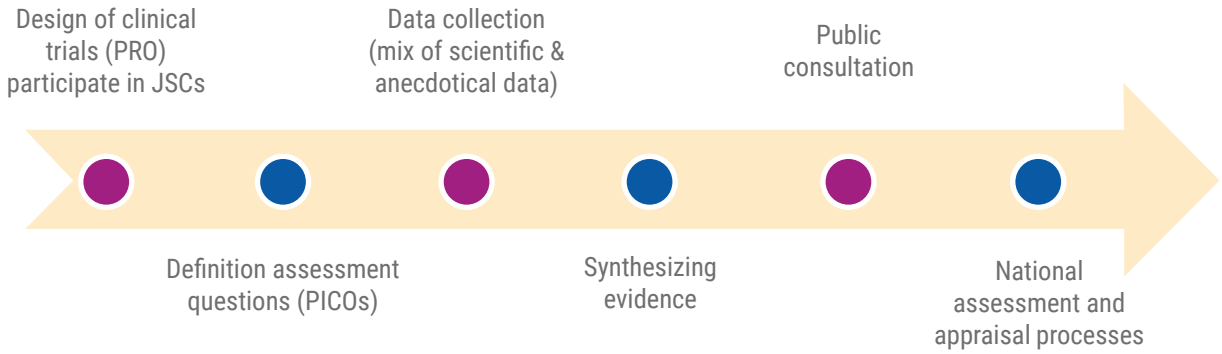
»»» **Figure 19. Patient participation must be ensured at all stages**



Source: adapted from EFCCA HTA Guidebook for patients (2024)[11].

in addressing those gaps from the start. Patient involvement, when integrated early, builds a culture of meaningful patient engagement, where patient insights continuously shape HTA standards.

Experts also highlighted the importance of involving patients at all phases of the medicine development, including the design of clinical trials, the definition of PICOS (Population, Interventions, Comparators, Outcomes, and Study Context)[20,21] the collection and synthesis of evidence, and the public consultations on different topics related with healthcare interventions. We may also be involved in national assessment and even appraisal processes, to make sure that our insights are considered at the decisions (»»» Figure 19).

5.2.6 Lack of transparency and conflict of interests

It is common for patient associations to collaborate with pharmaceutical companies, which is acceptable when done transparently and fairly. However, there is a concern that such partnerships might compromise the independence and credibility of patient organizations. In some European countries, there is skepticism about these collaborations, often viewing patient associations as influenced or sponsored by pharmaceutical companies. This perception can diminish the valuable role patient organizations play in advocating for patient interests.

The advice from the experts for EFCCA would be to maintain transparency and ensure that any collaboration is driven by genuine concerns and objectives of the patient community. It is important for patient organizations to assert their own voice and priorities while being open about their partnerships. While respecting confidentiality and personal data protection, making this information available for public viewing is crucial.

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Conflicts of interest in patient involvement can emerge from a range of sources, including financial relationships with the pharmaceutical or medical device industry, personal experiences that may bias the patient's perspective, or affiliations with organizations representing specific interests. Such conflicts have the potential to shape decisions in ways that may not fully reflect the diverse needs and concerns of the broader patient community, thereby undermining trust in both the evaluation outcomes and the healthcare system at large. There are also substantial differences in how "independence" is interpreted across contexts. For instance, in Germany and France, independence is often defined by the financial structure of patient associations, with an expectation that association budgets remain minimally reliant on pharmaceutical industry funding. Conversely, the UK adopts a more pragmatic approach: independence is understood as an absence of recent direct involvement, such as participation in advisory boards, with the specific pharmaceutical company associated with the dossier under review.

To ensure that patient contributions to healthcare assessments are both objective and equitable, it is essential to implement transparent mechanisms and training programs to identify, disclose, and manage these conflicts. Experts agree that nearly everyone has some level of conflict of interest, underscoring the importance of transparent disclosure practices. By declaring these relationships openly, we can foster trust and clarity, allowing conflicts to be addressed appropriately without compromising the integrity of patient involvement in healthcare decision-making. The European Commission has published rules of conflict of interest that must be applied to all individual experts including patients.

Patient associations should ensure transparency in their collaborations with pharmaceutical companies to maintain credibility. Conflicts of interest must be openly disclosed, and patient data should be publicly shared to improve healthcare assessments. Ongoing communication with regulators and industry stakeholders is key to building trust and accountability.

Ensuring transparency in data sharing and public access to information is also fundamental for fostering trust and accountability within healthcare decision-making processes. To advance this transparency, data from patient-generated focus groups, testimonials, and surveys should be made publicly accessible rather than restricted to internal use of patient

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associations. Although these sources may not be peer-reviewed, they may complement, if the relevant studies are rigorously carried out, the core evidence, thus contributing to the comprehensiveness of assessments.

Regulatory bodies also have a critical role in promoting transparency. However, transparency in the HTA and reimbursement processes varies significantly across countries. While countries like France, the United Kingdom, and Germany have taken steps to make these processes more publicly accessible, other countries are less forthcoming with information regarding drug assessments and reimbursement criteria decisions. In Spain, for instance, the proposed Royal Decree on HTA, currently in the draft stage, seeks to harmonize the Spanish HTA framework with EU standards. This decree also aims to establish an independent, transparent, and participatory system, which would not only enhance the clarity and accessibility of decision rationales but would also ensure that related documentation is made available to the public while maintaining commecial sensitive information confidential. In Italy steps have been taken towards a higher level of transparency in the assessment of drug innovativeness[23].

Lastly, requesting feedback from HTA bodies, regulators and industry stakeholders regarding patient contributions is a vital aspect. It would be advisable to establish ongoing communication channels to foster a relationship of trust and collaboration with regulators and industry partners.

»»» Figure 20. Recommendations to strengthen transparency and avoid conflicts of interest

Main issues	Recommendations
• Concerns about independence and credibility. • Variability in interpretation on independence. • Conflicts of interest from various sources. • Lack of transparency about process, timings and decisions. • Need for ongoing communication with regulators.	• Maintain transparency to ensure that any collaboration is driven by genuine concerns and objectives of the patient community. • Clear governance of the collaboration with the industry to ensure credibility. • Maintain the independence: assert your own voice and priorities while being open about partnerships. • Disseminate the results of surveys, focus groups and interviews. • Ask HTA bodies, regulators and industry for feedback about your contributions.

Source: own preparation.

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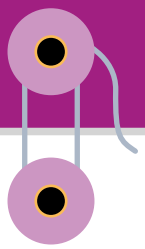

[CONCLUSIONS]

This pioneering initiative exemplifies how patient associations can take a proactive role in HTA. Unlike traditional models where experts primarily engage patients for their insights, EFCCA has taken the lead in collaborating with experts to improve the assessment of healthcare technologies for IBD. The collaboration with the Weber Foundation focused on fostering a stronger relationship between patients and European HTA bodies. The primary objective was to empower patients, equipping them with the authority and knowledge to represent their interests in a scientific manner.

To engage effectively in HTA processes and achieve better outcomes, patients and patient advocates can adopt several strategies (**Figure 21**). First, advocating for the inclusion of patient perspectives from the early stages of the technology lifecycle is essential. This involvement ensures that patient needs and experiences inform data collection, assessment, appraisal, and ultimately decision-making. Engaging early in the HTA process -through contributions to protocol design and outcome prioritization- helps shape assessments to reflect patient viewpoints from the outset.

Furthermore, developing a comprehensive understanding of HTA methodologies and processes is crucial. Patients should be trained to provide feedback that aligns with HTA and regulatory expectations, effectively combining empirical evidence with personal

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insights. Familiarity with key study design elements, such as the PICOS framework and indirect comparisons, will enhance patient contributions to discussions.

The initiative also seeks to clarify the specific information that should be communicated to ensure that the HTA process genuinely reflects patient concerns. Maintaining transparency and credibility within patient associations is vital for effective advocacy. By collectively promoting standardization and inclusivity in HTA processes, patients can play a pivotal role in shaping a healthcare system that is responsive to their needs.

HTA bodies across the EU are moving toward a more participative, transparent, consistent and evidence-based HTA framework. The ongoing regulatory evolution presents an opportunity to reshape patient involvement in HTA, fostering a more inclusive healthcare system for those living with IBD. However, it is essential to acknowledge that meaningful changes may take time. To realize this potential, all stakeholders must be prepared and united in pursuit of a common goal. As patient associations, we need to adapt to change by improving our skills, broadening our knowledge, and boosting our influence.

»»» Figure 21. Main messages from the meetings with experts

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