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# PATIENT ENGAGEMENT IN HEALTH TECHNOLOGY ASSESSMENT

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*Practices and Principles for Europe  
& the United States*





# INTRODUCTION

Amid a steady stream of new medications, diagnostics and medical devices, global policy conversations about the value of health technology are more important than ever.

Individual countries vary in their approach to health technology assessment. But one factor remains critical: patient engagement. No country or organisation can fully capture the value of new medications or medical devices without considering the end users.

Patient input allows assessments to incorporate the lived experiences of people with the disease treated by the medication or device being evaluated. It allows patient advocates to advise health technology organisations about the symptoms that most impact quality of life. Patients' experiences can also guide health technology organisations in judging the effectiveness of new treatments using factors that matter to patients.

Yet patient advocacy groups discern a gap between the input opportunities offered to them and the *impact* their input actually has.

Advocates may have the chance to speak at a public forum, for example, but not to partake in the decision-making process. They may be permitted to complete questionnaires but not see their responses meaningfully change the assessment methodology or approach. Advocates may have the opportunity to review an assessment report, but only once it's nearly finalised.

Beyond the chance to have a voice, patient advocacy groups are eager to see their experiences and insights incorporated in a more tangible way – informing the assessment process and, ultimately, helping to ensure more meaningful outcomes.



# EVALUATING PRACTICES IN EUROPE & THE UNITED STATES

To gauge how, and how well, different countries incorporate patient input, the Global Alliance for Patient Access reviewed health technology assessment practices across France, Germany, Italy, Spain, the United Kingdom and the United States. This report highlights those countries' relative strengths and weaknesses, assigning each country a letter grade based on its patient engagement.

## Patient Engagement Opportunities & Impact

<i>Grade:</i>	<i>A</i> - Robust	<i>C</i> - Needs Improvement
	<i>B</i> - Substantive	<i>D</i> - Unsatisfactory

Grades reflect adherence to best practices, specifically whether each country:



### Has a robust infrastructure for patient engagement.

- Does the assessment process include clearly defined opportunities for engagement?
- Does the organisation have staff, training or guides to facilitate engagement?
- Does the process allow sufficient notice and time for feedback?



### Makes materials intelligible and usable for patients.

- Does the organisation provide plain-language versions of reports and methodology?
- Does the organisation provide questionnaires to make commenting simpler?



### Empowers patients in the decision-making process.

- Are meetings transparent and open to patients?
- Do patients hold seats on committees and working groups?
- Do patients have voting rights in the decision-making body?
- Can patients appeal or challenge assessment decisions?



### Meaningfully incorporates patients' input.

- Do in-person proceedings reflect written input from patients?
- Are methodology and findings updated to incorporate patient feedback?
- Do health technology assessment organisations report how they will incorporate, or have incorporated, patients' feedback?

# UNITED KINGDOM

Health technology assessment in the United Kingdom primarily occurs through NICE, the National Institute for Health and Care Excellence.

NICE's Public Involvement Programme provides a format for patient organisations to participate in the assessment process. It proactively identifies and approaches patient organisations, rather than requiring them to locate input opportunities themselves, and it supports organisations participating in NICE's work. A formal policy on patient involvement undergirds the process.

As with other European nations, however, it is unclear in the U.K. whether patients have a meaningful impact on process or findings. Patients do not have voting rights in the health technology assessment process, and a 2018 study showed that committee members have mixed feelings about patients' input and participation. The same study found that patients often felt they lacked any meaningful influence on outcomes.<sup>1</sup>

## Infrastructure for Engagement

- ✓ NICE has a policy outlining its approach to patient involvement
- ✓ NICE proactively identifies and approaches patient and caregiver associations
- ✓ NICE's Public Involvement Programme provides direct support and training to patients and patient organisations

## Intelligible & Usable Materials

- ✓ NICE provides templates for patient organisations to submit evidence
- ✓ NICE offers a plain-language version of final reports for patients

## Patients Empowered in Decision-Making Process

- ✓ Patients can join committees or working groups
- ✗ Patient organisations have no voting rights
- ✗ Research suggests patients are underrepresented<sup>1</sup>

## Meaningful Incorporation of Feedback

- ✗ Committee members report mixed views, with some wary of patient narratives or critical of patient experts' contributions<sup>1</sup>
- ✗ NICE does not issue a formal report on how it incorporates patients' input

## Summary

The U.K. offers one of Europe's most formalised and structured approaches to engagement, but patient input does not always have a meaningful impact.

To better empower patient organisations, NICE could explain before an assessment how input will be used and, similarly, identify after an assessment's conclusion what impact patient advocates' input had. NICE might also look for additional opportunities to boost patient advocates' role in the decision-making process, such as by extending voting rights.

# GERMANY

In Germany, health technology assessment is undertaken by the Institute for Quality and Efficiency in Health Care, known as IQWiG, and the Federal Joint Committee, known as G-BA. At the G-BA's request, IQWiG assesses specific medications or diagnostics.

Germany is one of the few countries to include patient representatives on all committees and subcommittees, where five seats each are reserved for patients. Germany also offers financial and organisational support to patient groups that engage in the process.

When it comes to incorporating feedback, however, it's unclear whether patient organisations' input has a significant bearing on health technology assessment outcomes.

## Infrastructure for Engagement

- ✓ Financial and organisational support, such as assistance in preparing comments, is provided to patient organisations
- ✗ The comment period is only 2-3 weeks
- ✗ Outreach favors four large national patient organisations without clear opportunities for smaller groups<sup>2</sup>

## Intelligible & Usable Materials

- ✓ G-BA posts minutes of its public meeting online

## Patients Empowered in Decision-Making Process

- ✓ Patient representatives are members of the core committee, as well as of all subcommittees and working groups
- ✓ A public session is open to all groups that register
- ✗ The voting session is not open to the public
- ✗ Patient representatives cannot vote or challenge the decisions made

## Meaningful Incorporation of Feedback

- ✗ Germany does not issue a formal report on how it incorporates patients' input

## Summary

Patient engagement is interwoven into Germany's health technology assessment process in a number of important ways. Patient organisations receive support in preparing comments, and committees involve patient representatives as members.

For patient engagement to have the most meaningful impact on an informed process and outcome, Germany could extend the time period for public comment. The country could also better empower patient advocates by extending voting rights and allowing them to attend voting sessions, which are currently held in private. In addition, smaller patient organisations throughout the country might add valuable perspective if the opportunity to engage were also extended to them.

# FRANCE

France's health technology assessment organisation is the Commission de la Transparence.

The commission takes several steps to encourage patient engagement. Questionnaires and guides, for example, are available to simplify the input process. France also allows patients to hold seats on a health technology assessment decision-making committee.

France is one of the only countries to proactively report the level of patient engagement in its process and to quantify how that input informs the assessment. Those data suggest, however, that patients' input has very little impact on health technology assessment outcomes.

## Infrastructure for Engagement

- ✓ Guides are available to help patients provide input
- ✓ Feedback on input is provided to patient organisations
- ✗ Patient organisations have only 45 days to provide input

## Intelligible & Usable Materials

- ✓ Questionnaires are provided to simplify patients' input

## Patients Empowered in Decision-Making Process

- ✓ Three members of the Transparency Committee are designated to incorporate the patient perspective into the assessment

- ✗ Patient advocacy groups themselves cannot participate in assessment meetings

## Meaningful Incorporation of Feedback

- ✓ Officials produce a report summarising the level of patient input and where that input appears thereafter
- ✗ No assessment reports have mentioned the contribution of patient associations, according to the 2020 patient engagement report<sup>3</sup>
- ✗ Only about half of verbal consultations have mentioned patient organisations' input<sup>3</sup>

## Summary

France sets an example for other countries by proactively reporting on the level and impact of patient engagement. This information, however, suggests that engagement from patient organisations does not always have a meaningful impact. To make patient organisations more empowered participants, France could consider sharing decision-making power, such as by extending voting rights, or allowing patients advocacy groups to participate directly in meetings.

# UNITED STATES

While no government-sponsored health technology assessment organisation exists in the United States, a private group called the Institute for Clinical and Economic Review assesses new medications and devices. Conclusions from ICER, as the organization is known, can influence coverage decisions by both private and government health plans.

In recent years, ICER has attempted to improve its patient engagement process. In terms of meaningfully incorporating patients' input, however, ICER falls short. ICER's reports often allude to patient input without meaningfully altering methodology based on that input. In oral sessions, advocates may be allowed to speak but are not allowed to vote.

## Infrastructure for Engagement

- ✓ ICER allows for input at several phases of the assessment process
- ✓ Questionnaires can be used to simplify the patient input process

## Intelligible & Usable Materials

- ✗ ICER's reports are hundreds of pages long, written at a university reading level
- ✗ Simplified summaries of ICER's reports are available but only after the assessment process has concluded

## Patients Empowered in Decision-Making Process

- ✓ Patient advocates can sometimes participate in a policy roundtable
- ✓ Patients can register to speak at ICER's public meeting

- ✗ The roundtable discussion occurs only after voting has concluded
- ✗ Individual speaking slots are limited to five minutes
- ✗ Patients do not have voting rights

## Meaningful Incorporation of Feedback

- ✓ Most patients receive explanations of why their input was or was not included
- ✗ A review of ICER reports found that only 16% of patient advocates' comments are included in assessment reports<sup>4</sup>
- ✗ Patient advocates are half as likely as other stakeholders to have their comments included, according to a review of ICER reports<sup>4</sup>

## Summary

ICER has a clear and established process for patient engagement, but the opportunities provided do not always lead to meaningful use of patients' input. The organisation could improve patient engagement by providing materials better suited for a lay audience. To improve the impact of patient engagement, ICER could extend voting rights, allow roundtables featuring patients' insights to take place prior to voting and look for opportunities to quantifiably incorporate patients' values into the assessment.

# SPAIN

In Spain, a national agency and several regional organisations coexist and cooperate on health technology assessment.

A methodological framework supports patient engagement in health technology assessment, and six of Spain's eight regional agencies had begun implementing the framework as of 2017. At present, however, patients in Spain are often still limited in their opportunities to engage. Moreover, there is sparse evidence on how patient input is incorporated into the assessment process and outcomes.

## Infrastructure for Engagement

- ✓ A national declaration has urged a strategy for involving patients in the health technology process
- ✓ The Spanish Network of Agencies for Assessing National Health System Technologies and Performance has published a methodological framework for patient engagement, which six of the country's eight regional agencies have incorporated
- ✓ Input opportunities take a variety of forms, incorporating surveys, focus groups, and telephone or face-to-face interviews
- ✗ Assessment organisations struggle with recruitment and capacity building for patient engagement

## Intelligible & Usable Materials

- ✗ It is unclear whether plain-language materials are uniformly available

## Patients Empowered in Decision-Making Process

- ✓ Patients participate in some assessment organisations' expert panels

## Meaningful Incorporation of Feedback

- ✓ Surveys suggest that patients felt their opinion was considered during assessments
- ✗ The impact of patient engagement on reports was notably uneven<sup>5</sup>
- ✗ Contributions to some reports have been perceived as more limited by researchers<sup>5</sup>
- ✗ Spain does not issue a formal report on how it incorporates patients' input

## Summary

The methodological framework is an important step toward overcoming the challenges that stem from Spain's decentralised approach to health technology assessment. Plain-language materials, perhaps created in collaboration with patient advocacy groups, could help encourage more widespread involvement. As patient engagement becomes a standard part of health technology assessment across the country, advocates could also benefit from sharing decision-making responsibility, including voting rights and representation on assessment boards and committees.



# ITALY

In Italy, health technology assessment can occur at both the national and regional levels.

At the national level, the Italian Medicine Agency, known as AIFA, has engaged patients only in specific instances. Involving citizens and patients in decision-making processes has, however, been identified as a priority by the Istituto Superiore di Sanità, the leading technical scientific body of the Italian health care system.

On the regional level, only two regions of Italy are known to have structured patient engagement activities as part of their health technology assessment. In Veneto, for example, patient organisations are present at every commission responsible for health technology recommendations. In Emilia-Romagna, patient organisations are present at every health-related working group, with particular collaboration when rheumatic disorders and biosimilar medications are involved. At present, however, both patient engagement and health technology assessment itself are fragmented in Italy.

## Infrastructure for Engagement

- ✗ Engagement is deeply limited by the fragmented nature of health technology assessment in Italy

- ✓ In Italy's Emilia-Romagna region, patient organisations are present at every health-related working group

## Intelligible & Usable Materials

- ✗ It is unclear the extent to which materials are made accessible for patient organisations that do have the opportunity to engage

## Meaningful Incorporation of Feedback

- ✗ Italy does not issue a formal report on how it incorporates patients' input
- ✗ It is unclear how, or if, patient input impacts the assessment process or the final outcomes

## Patients Empowered in Decision-Making Process

- ✓ In Italy's Veneto region, patient organisations are present at every commission responsible for health technology recommendations

## Summary

Fragmentation serves as a barrier for many patient organisations that might otherwise engage in health technology assessment. Few clear opportunities currently exist to engage, and data quantifying how that engagement may impact the process or final outcomes is severely lacking. To encourage more widespread patient engagement, Italy could begin by committing to easy-to-understand materials for health technology assessments. Regions could also follow the example of Emilia-Romagna by incorporating patient organisations into assessment working groups.

## FINDINGS SUMMARY

Country	Grade	Successes	Ideas for Improvement
United Kingdom	<i>B</i>	<ul style="list-style-type: none"> <li>Well organised engagement opportunities</li> <li>Support &amp; outreach to patient organisations</li> </ul>	<ul style="list-style-type: none"> <li>Outline and report back on how patient input will be or has been used</li> <li>Empower patients more in the decision-making process</li> </ul>
Germany	<i>B-</i>	<ul style="list-style-type: none"> <li>Organisational and financial support to help patient groups engage</li> <li>Patient representation on working groups and committees</li> </ul>	<ul style="list-style-type: none"> <li>Bring patients into the decision-making process by allowing them to attend voting sessions and by extending voting rights to them</li> <li>Extend comment period and open participation opportunities to more patient groups</li> </ul>
France	<i>C+</i>	<ul style="list-style-type: none"> <li>Reports on patient engagement</li> <li>Quantifies how engagement informs the process</li> </ul>	<ul style="list-style-type: none"> <li>Empower patients by extending voting rights</li> <li>Identify more meaningful ways to incorporate patient feedback</li> </ul>
United States	<i>C</i>	<ul style="list-style-type: none"> <li>Clear and established process for engaging patients</li> <li>Speaking opportunities at public meetings</li> </ul>	<ul style="list-style-type: none"> <li>Plain-language materials that allow more patient groups to meaningfully engage</li> <li>Voting rights to give patients a role in the decision-making process</li> </ul>
Spain	<i>C-</i>	<ul style="list-style-type: none"> <li>Strategy for involving patients</li> <li>Methodological framework for engagement</li> </ul>	<ul style="list-style-type: none"> <li>Despite fragmentation, empower patients through widely available plain-language materials</li> <li>Allow patients more decision-making power through membership on committees and boards</li> </ul>
Italy	<i>D-</i>	<ul style="list-style-type: none"> <li>In certain regions, patients are well represented on assessment working groups and commissions</li> </ul>	<ul style="list-style-type: none"> <li>Encourage more widespread engagement through easy-to-understand materials and decision-making roles for assessments across the country</li> </ul>



# PRINCIPLES FOR IMPROVEMENT

How can health technology assessment organisations better engage patients and advocates? The key lies in establishing meaningful collaboration rather than token consultation.

Moving forward, advocates and assessment organisations should look to several key principles.



## **Health technology assessments must demonstrate meaningful incorporation of feedback.**

Patient groups' efforts are in vain if health technology assessment organisations don't incorporate input in a meaningful way. From the outset, health technology organisations should outline how they intend to use patient input. The organisations also should be held responsible, once the assessment is completed, for reporting how patient input was incorporated.



## **Engagement requires intelligible and usable materials.**

Inviting patient engagement is disingenuous if not accompanied by efforts to make methodologies, reports and meetings intelligible to non-economists and non-scientists. Earnest "translation" of health technology assessment materials into plain language lowers the barrier to entry for patients and patient advocacy groups who want to engage.



### **Meaningful input takes support, time and resources.**

Health technology organisations often encourage patient participation but leave the onus on the organisation to track, analyse, identify and communicate the information required. To optimise patient engagement, health technology assessment organisations must help bridge the gap. Questionnaires, guides, training and health technology assessment staff who can facilitate the process make participation more feasible. Patient advocacy groups also need sufficient time to provide feedback on drafts and prepare for in-person comments.



### **Transparency encourages a fair and balanced assessment process.**

As patients ultimately feel the impact of health technology assessment outcomes, they deserve transparency into the process. Fully disclosed methodology, transparent proceedings, visibility into the decision-making process and meeting that are open to the public are all necessary to make health technology assessment transparent for patients.



### **Patients deserve decision-making power and voting rights.**

While an open ear is encouraging, a seat at the table is even better. For patients to have an impact on health technology assessments, they need a meaningful role in the decision-making process. That includes membership on boards and committees as well as voting rights. Patients and patient advocacy organisations also need a method to appeal or dissent assessment decisions that are unsupported or do not adequately reflect patient input.

Generating reliable health technology assessment requires that patients and patient groups not only be included but also be properly equipped to engage and make meaningful contributions to the process. Voting rights and committee representation are important steps that European nations and the United States can take to ensure that patients' input has an impact.

Assessment organisations also must be held accountable for how they incorporate the input they receive from patient groups. Reporting and analysis of how patient input informs the methodology, process and final outcomes of health technology assessment is critical for improving patient engagement, across both Europe and the United States, in the years to come.



## METHODOLOGY

The Global Alliance for Patient Access partnered with Pharmerit - an OPEN Health company, to complete the research that informed this report. Those findings, along with citations for source materials, are available online.<sup>6</sup> Research built upon 2017-2018 research conducted by Pharmerit on behalf of the **European Federation of Neurological Associations**, which examined patient engagement opportunities for health technology assessments of neurological medications.<sup>7</sup>

Research considered health technology assessment in France, Germany, Italy, Spain, the United Kingdom and the United States. Methodology consisted of a comprehensive literature review to understand the current role and impact of patient engagement in health technology assessment decision-making. It also made use of the 2017-2018 expert interviews conducted in France, the U.K. and Germany as part of the original European Federation of Neurological Associations report.<sup>7</sup>

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### About the Global Alliance for Patient Access

The Global Alliance for Patient Access is an international platform for health care providers and patient advocates to inform policy dialogue about patient-centered care.

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