executive insight
HEALTHCARE CONSULTANTS

Patient evidence in HTA: Does it matter?

Results of global surveys of payers, patient experts trained on HTA and pharmaceutical company employees shine a light on how the use of patient evidence can be optimized to ensure the patient voice is heard in HTA/access decision-making

Executive Summary

Despite growing acceptance around the importance of patient input and evidence in healthcare decision-making overall, there still seems to be inconsistency when it comes to the acceptance of patient evidence in HTA/access decisions. In recent surveys of payers, patient experts trained on HTA and pharmaceutical company employees:

38%

of payers said that patient evidence is either "*often*" (13%) or "*always*" (25%) considered in HTA/access decisions in their country.

85%

of patient experts believed that patient evidence is not adequately considered in decision-making.

A key reason seems to be the persistent perception among many payers that patient evidence (e.g. PROs) is subjective and qualitative, while the HTA process is the opposite. Patient experts, pharma and payer survey respondents all agreed that patient evidence should be more systematically included in HTA/access decision-making, but how can this be achieved in a way that overcomes existing concerns?

The majority of survey respondents agreed that pharma needs to play a key role in facilitating the incorporation of patient evidence in decision-making. We have identified 5 ways for Pharma to elevate patient evidence to achieve more balanced HTA/access decision-making – in partnership with patient communities and HTA/access stakeholders:

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1. Educating and elevating

Collaborate with all key stakeholders (patients, patient organizations and HTA/ payers) to provide relevant, tailored education on patient evidence and its value.

2. Building trust

Raise awareness and build trust in patient evidence at the HTA/access level and support this through combined policy efforts on the value of patient evidence.

3. Generating robust evidence

Co-create patient-relevant PROs and other patient evidence methodologies based on what matters to patients and their unmet needs.

4. Enabling early co-creation

Ensure patients are involved early on and systematically to provide adequate time for the necessary co-creation.

5. Demonstrating impact

Show how integration of patient evidence in HTA/access decision-making leads to higher quality access/HTA decisions.

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Ensuring patients are involved earlier to develop more meaningful patient evidence for consideration at HTA/access decision-making will benefit all stakeholders. For pharma, clinical programs will be focused on what really matters to patients, increasing the likelihood of developing truly impactful treatments and meeting unmet needs. For payers, they will have a more complete picture of what constitutes value to patients, assisting with difficult decisions on spending priorities. And for patients, they will be empowered and ultimately benefit from improved access to potentially transformational medicines.

For all stakeholders,

… the systematic integration of patient evidence into HTA/access decision-making provides an opportunity to break down silos and build long lasting relationships so at last, when patients talk, they are truly heard.

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Introduction

In other industries outside of healthcare, it would seem strange not to get input from the end user of a product throughout development. But it is only relatively recently that pharmaceutical companies, regulators and payers have woken up to the idea that getting meaningful patient input throughout the product lifecycle improves outcomes for patients and society.

It is widely recognized that patients can bring unique perspectives, whether that's first-hand accounts of the symptoms and impact of a particular disease, their day-to-day experiences with therapies, or the management of side effects and the life-changing accommodations that are required to receive treatment.

But despite growing acceptance around the importance of incorporating patient evidence into healthcare decision-making overall, there still seems to be great inconsistency when it comes to its use in health technology assessment (HTA) / access decisions.

In this report, we present the findings from surveys of patient experts with knowledge of and trained on HTA, payers and pharma employees conducted by Executive Insight and merakoi, to identify the value of patient evidence in HTA/access, the barriers to incorporating the patient perspective more into decision-making and how to overcome these.

Patient evidence in drug development

In recent years, pharma companies have increasingly recognized the value of patient input and evidence in the drug development process, whether by patients contributing to clinical trial design, ensuring patient-friendly conduct of trials, assisting with evidence generation and advising as experts on the disease burden and treatment journey.

The role of patient input and PROs is on the rise

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And this engagement has been proven to be effective. In a 2018 report, the Economist Intelligence Unit compared 4,000 clinical trials with significant patient-centered elements with 20,000 traditional trials:

87%

of the patient-centric trials had positive results compared to …

68%

… of traditional trials. (The Economist Intelligence Unit, 2018)

The same study revealed that patient-centric trials took on average three months less time to recruit participants compared to traditional trials (The Economist Intelligence Unit, 2018).

In addition, real-world evidence is increasingly utilized today before and after approval to learn more about a product's performance outside the confines of a clinical trial.

A key reason why patient input seems to carry relatively little weight in HTA/access decision-making is probably the persistent view that patient evidence is subjective and qualitative, while the HTA process is the opposite. But the clear value of patient evidence in drug development is proof that patient input can be quantified and correctly utilized to the benefit of all.

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Patient evidence in drug regulation

The European Medicines Agency (EMA) and the US Food and Drug Administration (FDA) both consider that the voices of patients in medicines regulation are essential, as they bring the unique perspective of someone living with a disease, as a patient or caregiver (Mavris, 2019).

The 21st Century Cures Act, passed in the US in 2016, places additional focus on the use of patient-reported data, including real-world data, to support regulatory decision-making, including approval of new indications for approved drugs.

A section of the 21st Century Cures Act directs the FDA to report on the use of patient experience data in regulatory decision-making, especially focusing on the review of patient experience data and information on Patient-Focused Drug Development tools as part of applications.

Meanwhile, patient representatives are included in the EMA's management board and scientific committees, and are consulted by working parties in the preparation of guidelines. The EMA has also announced a new pilot initiative to enable early and systematic contact with relevant patient / consumer organizations at the start of each new product assessment.

Patient-Reported Outcomes (PROs) – a vital piece of the jigsaw

PROs – health outcomes directly reported by the patient who experienced them – are a key method of quantifying and objectivizing patient input at an early stage in clinical development.

As EUPATI has stated, "Clinical effectiveness measures cannot tell us how a patient feels or functions, or what they want to achieve from a treatment" (EUPATI Open Classroom). It is vital that evidence that

is most meaningful to patients is captured and reflected in the key decisions that impact the patient.

PROs "provide a patient perspective on a disease/treatment that might not be captured by a clinical measurement but may be as important to the patient." (EUPATI Open Classroom). PROs have helped pharma and medical device companies to understand the degree of disease severity from a patient's perspective, and now they are starting to become more widely recognized by payers worldwide as a key component of their decision-making process (Brogan, 2017).

"PROs provide a patient perspective on a disease/ treatment that might not be captured by a clinical measurement but may be as important to the patient."

Today, an increasing number of oncology medications for example, enter the market with product labeling claims that contain PRO data, meaning payers already need to familiarize themselves with the opportunities associated with PRO evidence when making coverage decisions (Zagadailov, 2013).

However, it is fair to say that historically PROs have sometimes been lacking in quality or robustness, especially in terms of critical methodological aspects of collection and analysis (Bylicki, 2015). Too often PROs are not disease-specific, have not been developed with patients, or are not really patient relevant – PROs have typically been developed by key opinion leaders who "knew their patients", rather than from understanding the direct experiences and perspectives of patients themselves. The use of traditional PROs assessing quality of life in Duchenne Muscular Dystrophy (DMD) and Multiple Sclerosis (MS) are examples where PROs have not adequately captured what is meaningful for patients, including e.g. DMD: access to care, emotional effects of disease, social skills, and caregiver impact (Bann, 2015); MS: emotional and daily life impacts, cognitive function, pain, recall period and scoring scale (Bharadia, 2021).

Those working in rare diseases have spearheaded the use of PROs, so it is unsurprising that some of the shortcomings of traditional PROs were first exposed in these spaces. It follows that rare diseases such as DMD have led the way with developing innovative and broadly validated PROs. Project HERCULES is a groundbreaking multinational

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collaboration set up by Duchenne UK to develop tools and evidence to support HTA and reimbursement decisions in DMD (Duchenne UK). One of the most impressive outcomes is the development of DMD-QoL, a new bespoke and validated Quality of Life measure specifically for DMD (Powell, 2021).

Successes like these have been central to the movement towards getting more patient-centered measurements incorporated within clinical trials. Now can patient-centered measurements gain wider recognition with HTA/access decision-makers?

Case study: SMA Independence Scale

The SMA Independence Scale (SMAIS) measures the amount of assistance required by people living with spinal muscular atrophy (SMA) to perform typical daily activities, such as getting dressed or self-feeding (Genentech). This PRO was co-created early on with the SMA Community following qualitative research identifying independence as a key desire of the SMA community. The SMAIS was integrated into the clinical development program of risdiplam to generate critical evidence that can support broader patient access.

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Patient evidence in HTA/access decision-making

The use of patient evidence in health technology assessment (HTA) is becoming more frequent. However, there is "great variability and an absence of comprehensive, robust practices" (Wale, 2021).

	 Germany	 Italy	 England
Type of HTA	**Comparative clinical benefit driven**	**Mixed clinical benefit & budget impact driven**	**Cost effectiveness driven**
Formal process to include patient input	**Yes** – patient groups can provide **input on** IQWiG **assessment & comment** on assessment result, and **participate in G-BA hearing**, although with **no decision-making power**	**Limited** – generally the patient voice **is not included in the HTA process** – only in rare cases (although growing) and typically in rare diseases	**Yes** – patients are encouraged to **provide input in** & be a **part of appraisal discussions** and can **appeal against final recommendations** (greater input in highly specialized technology)
Extent that patient evidence is considered	> **Translation** of clinical outcomes into **patient relevant benefit** using **validated tools** is key > Data must be '**patient relevant**' (i.e. surrogates are not preferred) > Statements during the **commenting & hearing procedure** can **impact the overall result** (i.e. high unmet need conditions)	> **Priority** given to **patient relevant and clinically recognized data** (e.g. supported by guidelines) > **Greater weighting on primary endpoints** (e.g. typically favoring efficacy and safety data over PROs)	> Data must be **patient relevant**; overall **clinical & cost-effectiveness** typically **overshadow** patient perspectives > However, in **high unmet need** conditions (i.e. highly specialized technology) patient perspectives play a **greater role**

Based on internal Executive Insight knowledge

Generic PROs about health-related quality of life (HRQoL) for example, are not used consistently in HTAs across countries. In some countries, such as England, generic PROs are considered as part of the cost-effectiveness analyses. But in France and Germany, HRQoL is treated as an independent assessment criterion, which almost gives it equal weight to clinical evidence. Even then, strict data quality requirements often result in PRO evidence not being included or considered robust enough for assessment purposes.

The extent to which patient input is considered in HTA decision-making varies widely

Disease-specific PROs are also considered relevant evidence but are not used as often (and likely do not exist for all diseases) and currently tend to have less impact on the assessment decision. When disease-specific PROs are recognized, it is often PROs that are internationally recognized and validated, and that correlate with health outcomes from clinical data. Occasionally non-validated disease-specific PROs are considered for very rare conditions.

Some HTA agencies – such as NICE in the UK, IQWiG/G-BA in Germany, CADTH in Canada and HAS in France – are expanding their focus on patient-centered outcomes and patient experience data. In the UK for example, proposed process changes by NICE include elements to more systematically incorporate the patient voice and an acknowledgement that PROs "can capture important aspects of conditions and interventions" and should be "appropriately validated" (NICE, 2020).

In Canada, CADTH regularly considers multiple dimensions of patient preferences — including patient expectations, satisfaction, and views on different clinical endpoints (CADTH, 2021).

In France, as part of the 2021 early access program reform, HAS recommends that pharmaceutical companies integrate patient-relevant PROs – defined with patient organizations – as part of the program's real world evidence/data collection framework (HAS, 2021). These examples highlight the increasing role of patient evidence in HTA/access is gaining momentum.

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However, even in countries that are progressive in their use of PROs, doubts remain about the actual impact on HTA outcomes, and in general, patient evidence has still not achieved mainstream use or acceptance in HTA/access decision-making.

Why is this, what are the barriers to the use of patient evidence in HTA/access decision-making, and how can these be overcome? Executive Insight and merakoi conducted surveys of payers, patient experts and pharmaceutical company employees to find out.

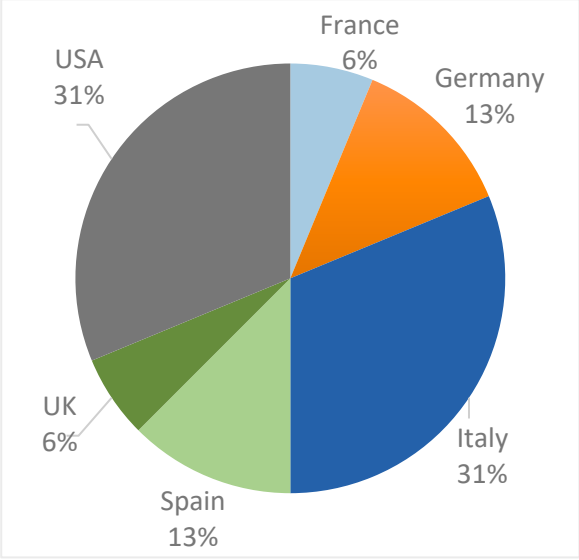
Methodology

Three surveys were conducted among payers, patient experts with knowledge of and trained on HTA and pharma employees to gauge opinions on the use of patient evidence in HTA/access decision-making.

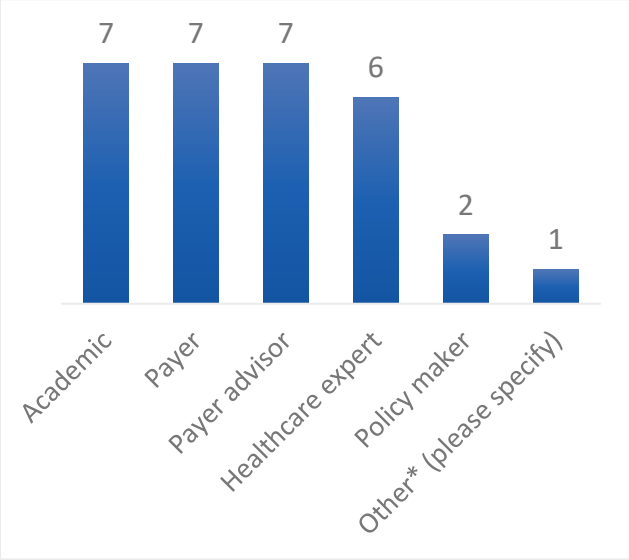
Payer survey

Executive Insight conducted the payer survey via an online questionnaire made available on 24th July until 6th August 2021. A total of 16 payers completed the questionnaire, 11 from Europe and 5 from the US. More detail on geographic distribution and participants' current or previous role can be found in the graphs below.

Payer participants: Geographic distribution (n=16)



Payer participant's current or most recent role (more than one possible answer)

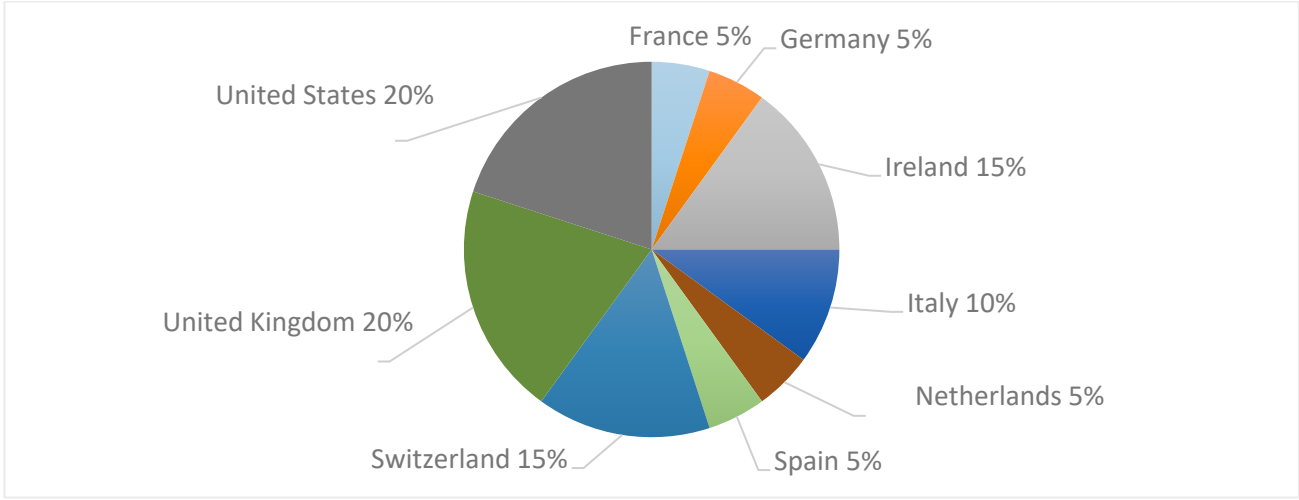


** Other: "HTA"*

Patient expert survey

merakoi conducted the patient expert survey via an online questionnaire between 14th June and 9th July 2021. A total of 20 patient experts trained on HTA completed the survey, 16 from Europe and 4 from the US (see geographic distribution below).

Patient expert participants: Geographic distribution (n=20)



Pharma survey

Executive Insight conducted the pharma survey via three polls published on the Executive Insight LinkedIn page between 25th May and 8th June 2021.

24 pharma respondents answered the first poll, 17 for the second and 14 for the third. Respondents were from a range of geographies but exact locations for all cannot be determined.

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Survey results

i. Patient Involvement in HTA/access decision-making

"Patients have perspectives and experiences that can uniquely contribute to improving the quality, relevance and value of the decision-making process. Fundamentally, patients should have the same rights to contribute to HTA as other stakeholders."

Patient Expert, Ireland

While many believe that patient evidence should be assessed in a more systematic way in the HTA/access decision-making process, the survey revealed this is currently not always the case.

Payers were asked to what extent patient evidence is formally considered in HTA/access decision-making. Only 25% of respondents said that patient evidence is always considered in their country, suggesting the incorporation of patient evidence into HTA/access decision-making is currently far from systematic.

HTA processes do not systematically consider patient evidence – at best it has an ancillary role

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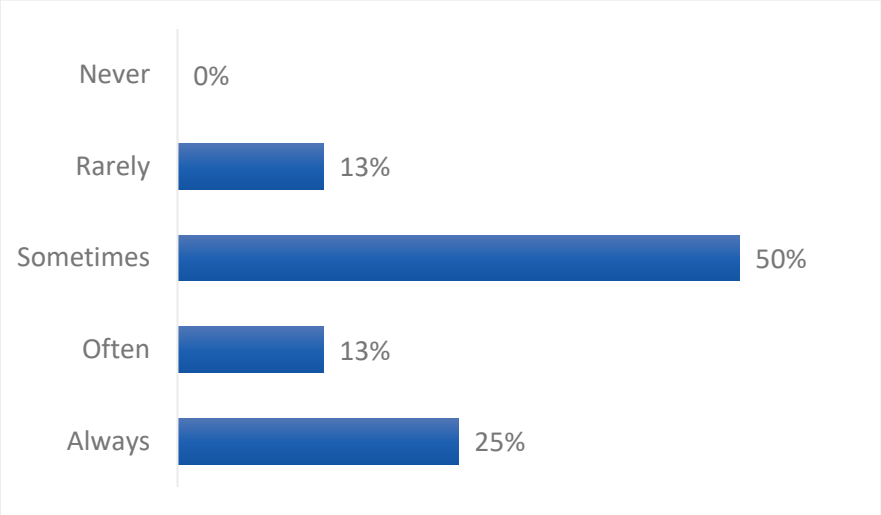
Payers: To what extent is patient evidence formally considered in HTA/access decision-making in your country today? (n=16)



"*Patient evidence is not contemplated in the formal evaluation procedures. Only quality of life results are taken into account in some cases*," said one Spanish payer. It is important that patient engagement in HTA/access can go beyond "token" activities and ensure the integration of meaningful patient data into deliberative processes.

The majority (85%) of patients in the survey also felt that patient evidence is inadequately considered in HTA/access decision-making. Even when patient evidence is considered, it is often not given the same weight as other evidence. Of the payers in the survey who stated that the HTA/access decision-making in their country considers patient evidence, the vast majority (86%) agreed that it has lower weighting compared to traditional clinical endpoint evidence. The payers in the survey suggested that the main use of patient evidence was for assessing product differentiation, which again suggests patient evidence as an optional extra to be used only in certain circumstances. Half (50%) of payer respondents said patient evidence was rarely or never used for reimbursement or pricing decisions.

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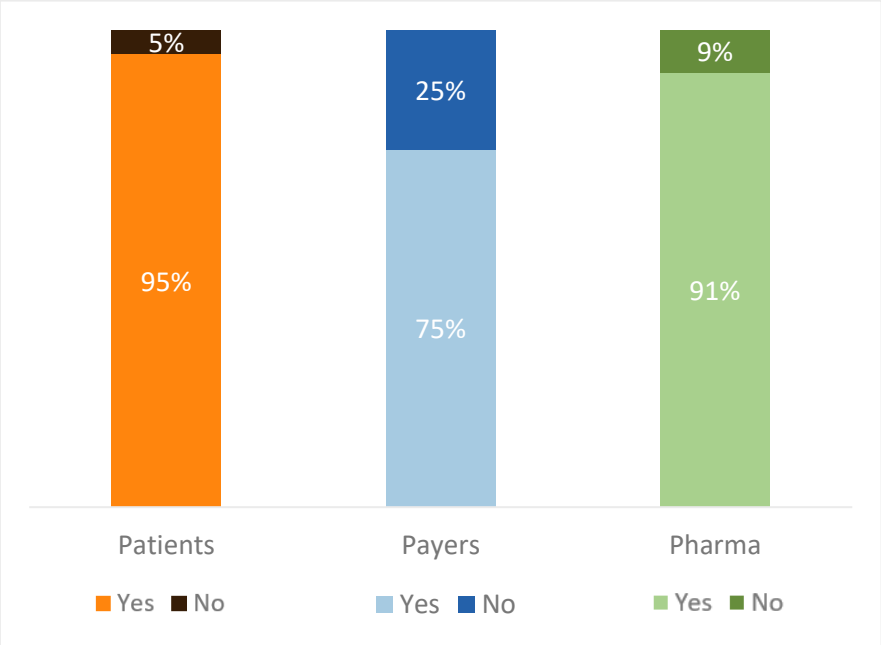
The good news is that the will for change appears to be there. The vast majority of all stakeholders in the survey believe that patient evidence should be included more systematically in HTA/access decision-making.

All: Should patient evidence be included more systematically in HTA/access decision-making in your country?



Although the proportion of payers agreeing with this statement (75%) was lower than the proportion of patients (95%) or pharma employees (91%), it still suggests there is overwhelming belief in the value of incorporating the patient perspective in systematically.

ii. Assessing the value of patient evidence

"Patient engagement is one of the key factors in order to better assess the value of different health care technologies and to improve the practical implementation of the HTA result in clinical practice."

Payer, Italy

Payers and patients are broadly aligned on the value of patient evidence, but disagree on its role in balancing HTA decision-making

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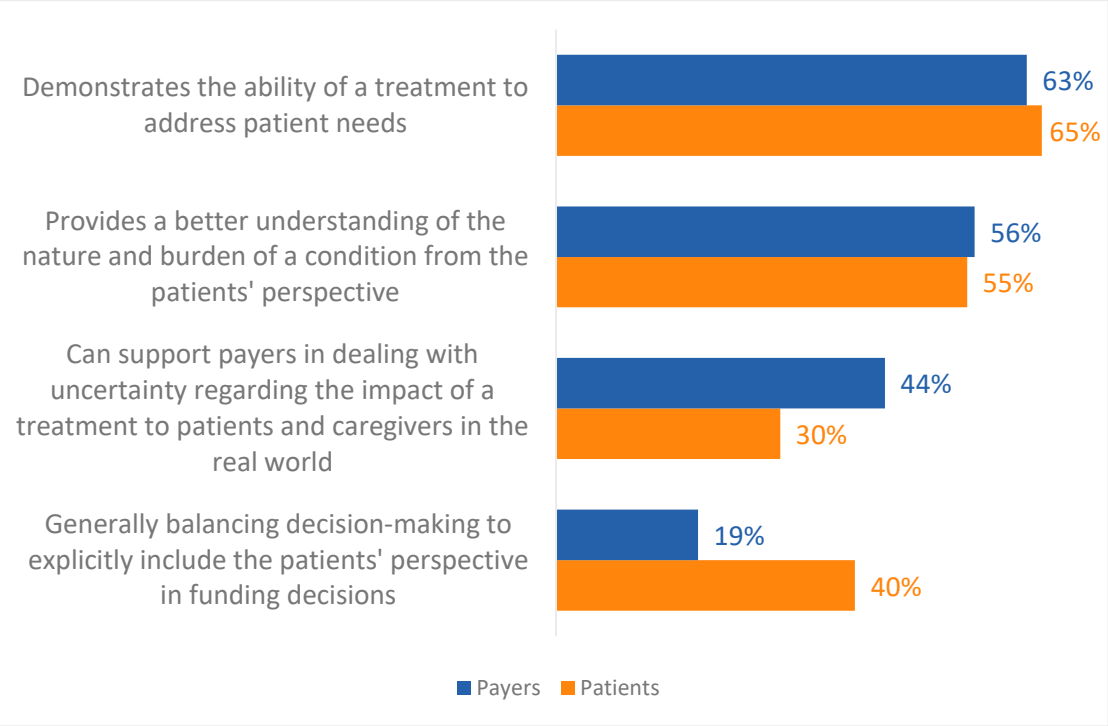
Payers and patient experts were asked about the value of considering patient evidence in HTA/access decision-making.

What do you see as the potential value of incorporating patient evidence in HTA/access decision-making?



Patient experts and payers were broadly aligned with the value that patient evidence brings, with over half of both surveyed stakeholder groups agreeing that patient evidence can demonstrate the ability of a treatment to address patient needs and provide an understanding of the burden of a condition from the patients' perspective.

"*A patient knows their body best,*" said one patient expert from the USA. "*How they feel on a particular treatment, their energy level, mood, quality of life, and so on – these are all important patient reported outcomes, which should be its own data set in assessing success of a given treatment. PROs still have not achieved mainstream respect and acceptance in metrics.*"

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A payer from the US summarized *that "The reason for healthcare is patient wellbeing. We should be more attuned to what really matters to patients."*

Meanwhile, only 19% of payers agreed that patient evidence can help to balance decision-making in funding decisions, again hinting at the relatively low weight given to patient evidence when it comes to the most important decisions made by HTA/access decision-makers.

40% of the patient experts thought that patient evidence can help to balance decision-making. *"It is not only the patients who live with a condition, but who also live with the consequences of treatment decisions,"* said one patient expert from the UK. *"As that decision (benefit-risk ratio) is at the core of HTA, then it follows that the patient experience of illness and treatment should also be in the center of HTA decisions."*

So, there is broad agreement that patient evidence has value – but disparity on exactly how much and for what.

iii. Barriers to making inclusion of patient evidence in HTA/access more systematic

"We need to quantify more the grade, to have more objective parameters, validated scales and to reduce the subjectivity of patients and researchers in the clinical studies."

Payer, Spain

More countries are actively progressing towards incorporating the patient voice in formal HTA. Among the payers in the survey, more than half (56%) were aware of specific initiatives in their country to ensure more systematic inclusion of patient evidence in HTA/access decision-making. All surveyed payers believed that these ongoing initiatives will have an impact in increasing the weight of patient

Payers perceive patient evidence as subjective and struggle to integrate it with other types of evidence

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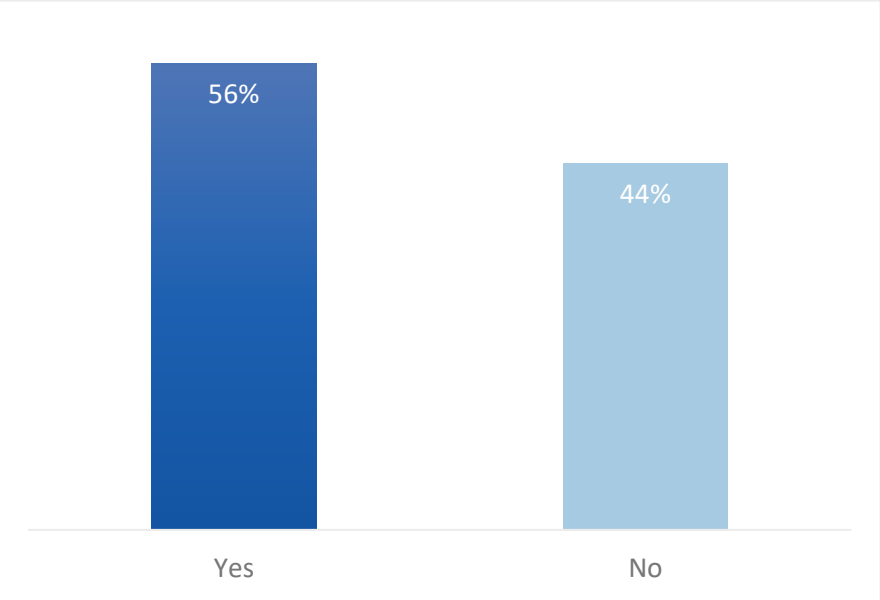
evidence in the HTA/access decision-making within the next three years.

Payers: Are you aware of specific initiatives in your country to ensure more systematic inclusion of patient evidence in HTA/access decision-making?



Italy

Italian payers highlighted that patient engagement is now required within the latest guidelines for compiling a HTA dossier. Patient involvement is envisaged within the documents of the National Medicines Agency (AIFA) and the Ministry of Health. Also, in Italy initiatives are ongoing to run training programs and include patient representatives in regional and national committees.

Spain

In Spain, payers mentioned that a new process is being established to include PRO data when evaluating new treatments, as well as PRO projects by the Spanish Society of Hospital Pharmacy. Projects are also ongoing to develop methodologies that can facilitate the integration of patient evidence into the overall decision-making process (multi-criteria decision analysis).

USA

Meanwhile, payers in the US highlighted that the FDA is attempting to include more patient centric approaches to drug development and regulatory approval, and to make more patient evidence available to other decision-makers. ICER is also developing more patient-centric approaches in cost-effectiveness analysis and value assessment.

Both patient experts and payers were asked what they see as the main barriers are to more systematic consideration of patient evidence in HTA/access decision-making. The results showed interesting differences in the perceptions of both stakeholders.

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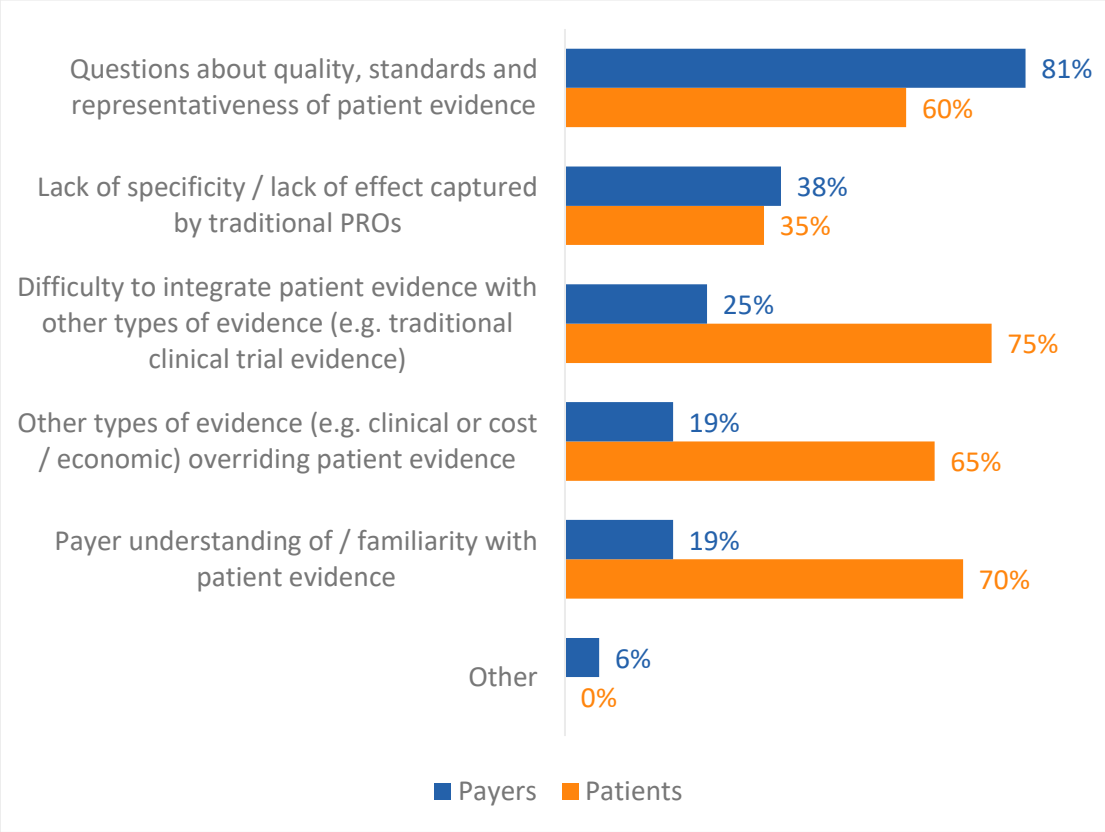
Patient experts and payers: What do you see as the main barriers to a broader and more systematic consideration of patient evidence in HTA/access decision-making?



**Other: "Patient representative organizations are a problem. Who do they represent and who funds them?"*

For payers, the most popular response was the issue of quality, standards and representativeness of patient evidence – or in the words of one payer from the US, "*patient evidence is not considered because it is viewed to be too subjective*". This seems to be a commonly held view, which reinforces the need to make the process more systematic and objective. "*From a clinical point of view, patient evidence is very heterogeneous … unless patient evidence is extrapolated alongside a clinical trial where the design and the randomization procedures are appropriate*," said another payer from Italy.

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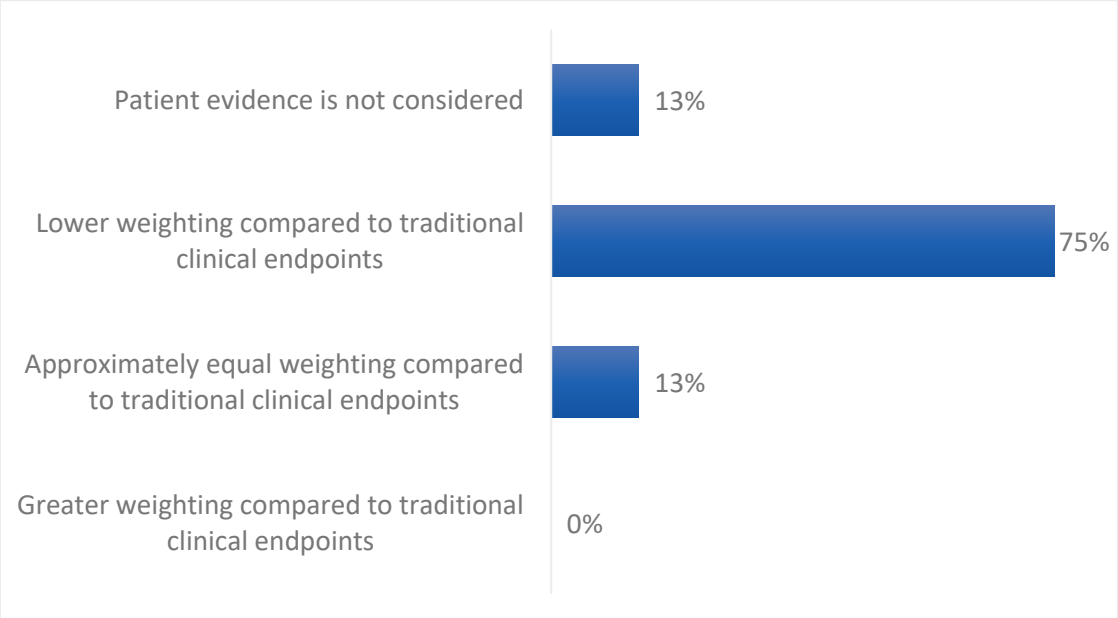
Interestingly, 60% of patient experts agreed that questions around the quality, standards and representativeness of patient evidence is a key barrier, although the barriers selected most by patient experts were the difficulty to integrate patient evidence with other types of evidence (selected by 75% of patient experts vs only 19% of payers) and payer understanding of / familiarity with patient evidence (selected by 70% of patient experts vs only 19% of payers).

One patient expert, from Italy, went as far as to say that there is "*a large ignorance by the regulators and payers of the impact of a disease on a patient. They are taking their decisions on a simple ratio cost/benefit based only on clinical outcome and not patient outcome.*"

> *"Regulators and payers take decisions on a simple ratio cost/benefit based only on clinical outcome and not on patient outcome"*

Perhaps unsurprisingly given their misgivings over its quality, payers said they considered patient evidence to a lesser extent than traditional clinical endpoints.

Payers: In your country, to what extent is patient evidence typically considered vs. traditional clinical trial endpoints (e.g. overall survival) as part of HTA/access decision-making?



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"AIFA, the Italian Government Agency for Medicines, historically prefers more objective data from the investigator, as it is considered more reliable," said one payer from Italy.

Where it is accepted, it is generally seen as complementary and secondary to other types of evidence.

"Patient evidence is a complementary information to RCT, it does not compile nor substitute to RCT," said a payer from Spain. *"In case of similar efficacy and safety, patient evidence has an important role."*

A payer from the US made the point that *"it varies considerably by therapeutic category"*. When asked in which disease area(s) patient evidence is considered more or has more weight in HTA/ access decision-making compared to others, payers predominantly mentioned oncology and rare diseases. One US payer said, *"patient evidence has more weight in oncology relative to other categories, however, it is still weighted significantly less relative to clinical endpoints."* Payers also mentioned that for pain and neuropsychiatric disorders (e.g. migraine, depression etc.) patient evidence is more compelling given limited clinical endpoints to measure an effect.

In most cases, *"the more objective endpoints such as survival are considered most heavily, and patient reported evidence plays only a supporting role to reinforce the other findings".*

iv. Overcoming barriers to incorporation of patient evidence in HTA/access decision-making

"Make sure patient evidence is included in all studies and have patients involved from the beginning in order to make sure that evidence collected is really relevant to patients. It shouldn't be clinicians to decide which PROs to include, but rather patients."

Patient expert, Italy

All three key stakeholder groups – patients, pharma and payers – agreed that patient evidence should be more systematically included in HTA/access decision-making, but how can this be achieved?

Policy change is needed to encourage consideration and appropriate weighting of patient evidence

Policy change as a priority

Given five options to choose from as the most important points to increase the role of patient evidence in HTA/access decision-making, the option selected most often by payers was policy change on the type and weighting of inputs considered.

Interestingly, only two payers agreed that having direct patient representation in the decision-making process was one of the most important points.

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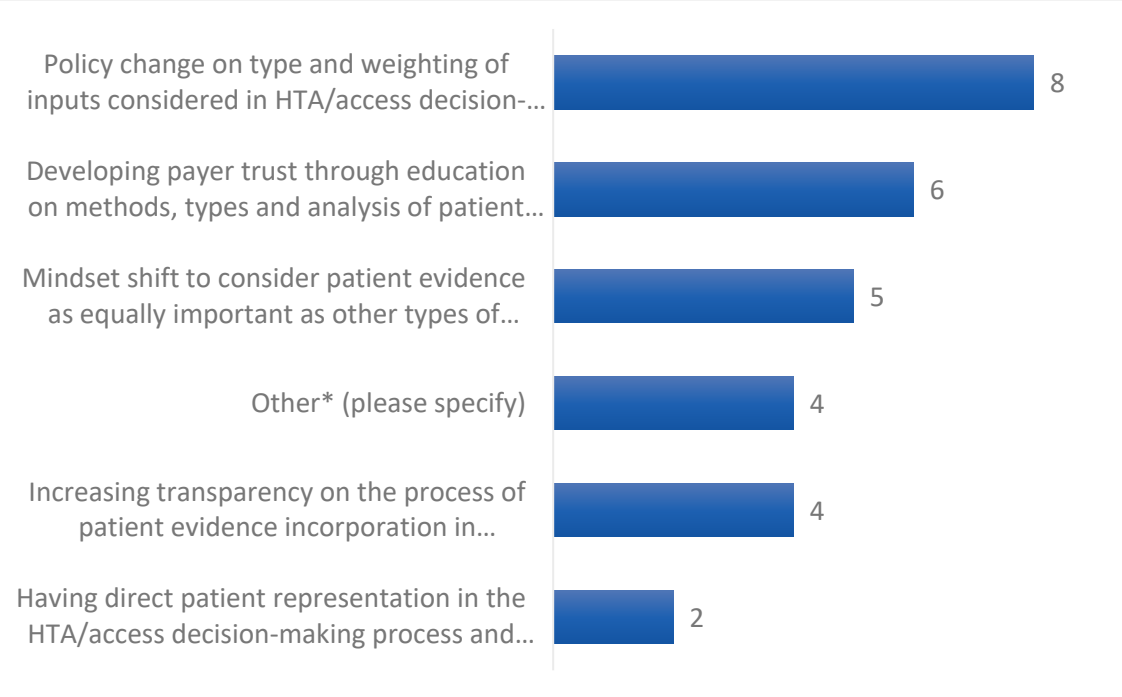
Payers: For your country, what do you see as the top 2 most important points to increase the role of patient evidence in HTA/access decision-making?



**Other:*

- *"Collection of valid data by companies with accepted instruments"*
- *"Having reliable and transparent patient organizations/networks"*
- *"Development/inclusion of validated specific PROs"*
- *"Providing robust evidence on the metrological quality of PROs"*

Role of patient organizations

When it comes to the role of patient organizations, over half of payers and patient experts (63% and 55% respectively) agreed that they should have an active role in patient evidence definition to ensure measures are specific and relevant and reflect what matters to patients. Over half (55%) of the patient experts believed patient organizations should have an active dialogue with payers to overcome perceptions of patient evidence being anecdotal, something which 38% of payers agreed with.

Collaboration needs to be increased and start earlier to give patients and patient organizations more influence in the development of PROs that matter

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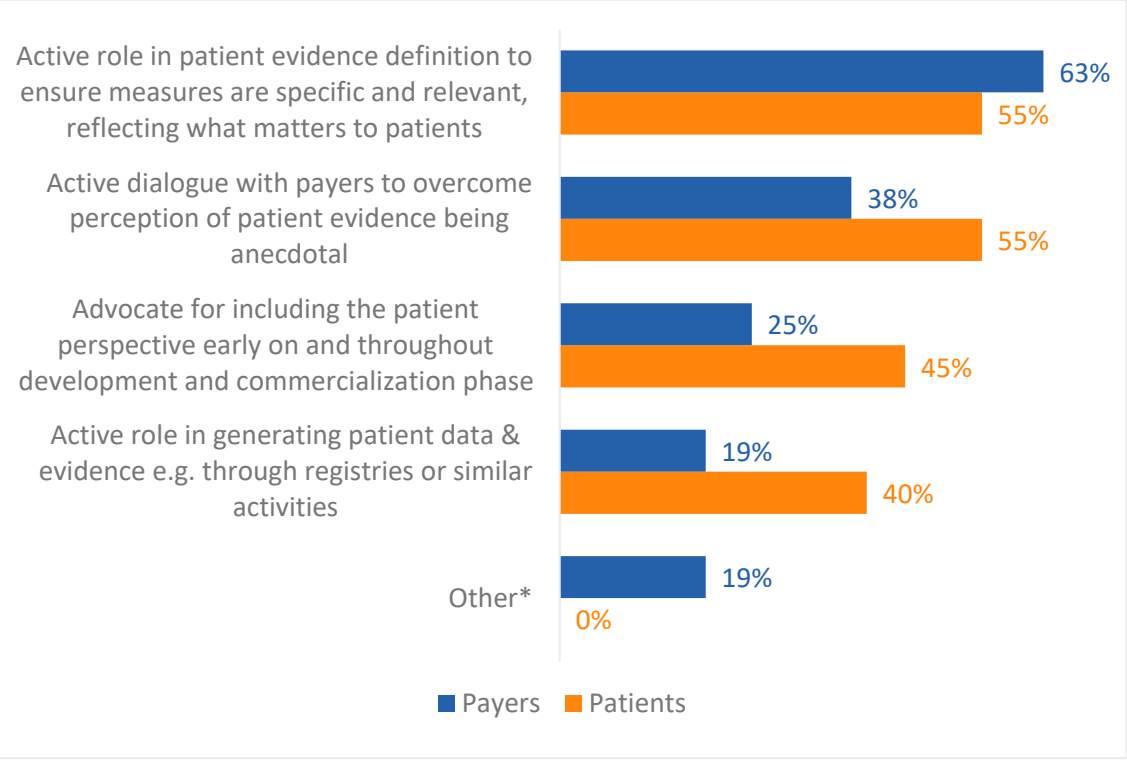
The role of patient organisations

Nearly half of patient experts believed patient organizations should advocate for including the patient perspective early on and throughout the development and commercialization phases.

Payers and patients: In your country, what do you see as the specific role of patient organizations in patient evidence generation?



**Other:*

- *"No direct roles, only lobbying"*
- *"Post-progression data is useful"*
- *"Not aware of any"*

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Role of pharmaceutical industry

All of the patient experts in the survey agreed that pharma needs to play a key role in facilitating the incorporation of patient evidence in decision-making. This could include ensuring PROs are co-created early on with patients to be integrated into clinical trials, gaining patient input early in trial design, validating patient evidence methodologies and publishing patient evidence together with clinical data.

"If pharma can work with patients to understand the experience of taking a new treatment – and can understand the side effects, the concerns, the potential issues – and address them before they get to HTA, then they look pro-active and patient-focused," said one patient expert from the UK. "*As pharma, for so long, has been complicit in this idea that patient evidence and experience is 'anecdotal' and meaningless, hard to understand and difficult to use, they now have a duty surely to rectify this. They can work with their sales teams, their market access people to raise the profile of patients and the importance of their experience as a key piece of evidence necessary for pharma development going forward*," said the UK patient expert.

The consensus seems to be that collection of patient evidence should happen early. EUPATI guidance states that the pharmaceutical industry should "*strive to involve patients early in medicines development, preferably before the clinical development phases*." After this point, *"many key decisions about a medicine have already been taken and cannot be reversed*" (Warner, 2018).

The majority of payers (69%) in the survey also supported the pharmaceutical industry's active role in facilitating the incorporation of patient evidence, although some have concerns around lack of transparency, conflict of interest and biased collection/interpretation of evidence. *"Decision-makers could misinterpret this approach,"* said one.

Pharma to integrate the patient perspective early on and systematically in product development and throughout the lifecycle

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Role of payers

The onus is on payers meanwhile to develop models and methodologies that can integrate multiple evidence inputs (clinical, economic, patient etc.) and come to balanced decisions. There must be the will and the means to ensure patients have a seat at the table and that their input is properly integrated and considered in access decision-making.

"*As the people who make the decisions about paying for a new medicine or not, they must consider all of the evidence available to them - and that increasingly should now contain patient experiences*," said a UK patient expert. "*Now they have to get closer to patients … and make their decisions based on better understanding of the end user. I believe they also have a duty to educate their population about how decisions are made*."

In terms of current patient involvement in HTA, one payer from France made the point that although patients "*sit in the transparency commission, the transcript of the verbatim of the meetings shows that the patients seldom or never speak*."

In contrast, the overall view of patient experts in the survey was that being involved in the HTA process is very important to them and patients feel that HTA/access decision-makers should involve them earlier and more systematically. "*The most important value of an HTA is that it is organised around and responsive to the needs of the people using it*," said a patient expert from the UK. "*The most effective way to ascertain this information is to consult and involve the users.*"

Moving forward and alongside integrating patient evidence in decision-making, payers should look to include patients more systematically in their HTA/access processes. Pharmaceutical companies could play a role here by providing training and capability building to enable patients to contribute objectively and meaningfully – and to feel comfortable in doing so!

Payers to integrate patient evidence with other evidence types to enable balanced decision-making and more systematically include patients in their processes

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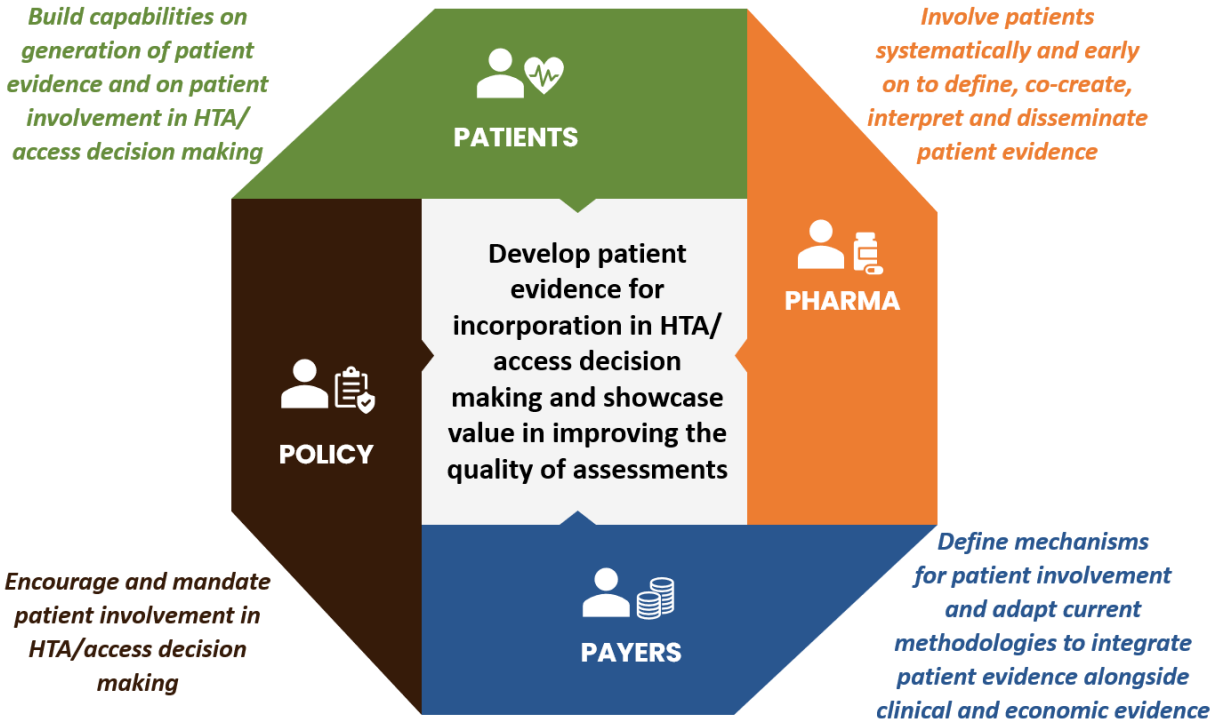
Moving towards earlier, more systematic patient involvement & evidence

To ensure that patient input is systematically included in more HTA/access decision-making, all key stakeholders – pharma, payers and patients alike – have a role to play. Genuine co-creation and open lines of communication are vital. It is important that at an early stage, all three stakeholders align on what meaningful patient evidence looks like and how to incorporate it into HTA/access. Moving forward, healthcare system policy change is also likely required to achieve systematic integration. The below image shows the potential roles of each key stakeholder in enhancing the incorporation of patient evidence in HTA/access decision-making.

Realistically, as pharma are the 'owners' of the product lifecycle, they need to be in the driving seat when it comes to proactively setting up multi-stakeholder initiatives and alignment. As one US payer put it: "*They fund and conduct the clinical trials – in the absence of pharma, it would be difficult to see a path forward where another entity will step in to generate this type of evidence*".



Implications – what does this mean for Pharma?

We have identified 5 ways for Pharma to elevate patient evidence to achieve more balanced HTA/access decision-making – in partnership with patient communities and HTA/access stakeholders. As a foundation, it is essential that pharma companies have the right capabilities in place across their whole organizations to achieve the following:

1.

Educating and elevating

Collaborate with all key stakeholders (patients, patient organizations and HTA/ payers) to provide relevant, tailored education on patient evidence and its value. Partner with patient organizations to build their capabilities and train patients to meaningfully contribute in HTA/access meetings and in co-creation with pharma. Leverage the general training materials and resources from EUPATI and PFMD as a starting point in the education process with specific, tailored training at a disease level as the ultimate aim.

How to:

Create a capability building pilot with a trusted patient organization. Together, assess the HTA education level of members in the patient organization to co-define a training curriculum that can effectively fill the gaps.

Develop an aligned cross-functional position within your organization on patient evidence to amplify a clear message in payer interactions – as part of broader environmental transformation efforts.

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2.

Building trust

Raise awareness and build trust in patient evidence at the HTA/access level and support this through combined policy efforts on the value of patient evidence. Use existing initiatives from organizations such as European Patients Forum, Eurordis, EFPIA and PhRMA as a foundation to build on and amplify.

How to:

Define a compelling outside-in vision for patient evidence in HTA/access across the organization as a critical starting point for aligned external communications.

Transform the environment through tailored multi-stakeholder educational and policy initiatives based on an assessment of the current landscape and perception.

3.

Generating robust evidence

Co-create patient-relevant PROs and other patient evidence methodologies based on what matters to patients, their unmet needs and their perception of the issues with current treatments. Ensure methodologies are validated by payers and other key stakeholders for use in clinical programs – ensuring evidence generated is accepted later in HTA/access.

How to:

Develop disease specific PROs with the patient community well ahead of the pivotal trial – starting from a conceptual model of disease based on patients' perception of disease burden and impact in real life and determining what is critical to measure and how.

Develop an early product strategy accounting for all stakeholder needs and identifying what type of patient evidence would be most meaningful and how to optimally measure this.

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4.

Enabling early co-creation

Ensure patients are involved early on and systematically to provide adequate time for the necessary co-creation. Consult with payers and other key stakeholders early on too and regularly throughout the co-creation process to gain their input. Effective co-creation with all stakeholders takes time!

How to:

Instill a patient first and inclusive mindset throughout the entire organization, enabled through the right tools and resources and backed up by the necessary capacity for early patient engagements.

Establish a transparent, cross-functional operating model and ways of working to ensure a consistent, positive collaboration experience for patients, and to effectively integrate the patient voice across the whole organization.

5.

Demonstrating impact

Show how integration of patient evidence in HTA/access decision-making leads to higher quality access/HTA decisions.

How to:

Identify best practice examples as ‘proofs of concept’ to concretely demonstrate the value of patient evidence in HTA/access decision-making to enable more systematic implementation by healthcare systems.

Develop an internal measurement methodology focused on tracking the impact of integrating the patient perspective/patient evidence to facilitate systematic inclusion across the organization – set-up a pilot as a first step upscaling.

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Looking ahead

Taken together, our results highlight a clear readiness and willingness by all stakeholders to establish a healthcare system level measurement system to track the extent of patient and societal benefit of new treatments and solutions. Implementing such a tracking system requires close partnering between all stakeholders and would further help to incentivize all stakeholders towards what is meaningful for patients and beneficial for healthcare systems, while also supporting sustainability.

Ensuring patients are involved earlier to develop more meaningful patient evidence for consideration at HTA/access decision-making will benefit all stakeholders. For pharma, clinical programs will be focused on what really matters to patients, accelerating clinical trial enrolment and generating meaningful evidence, and ultimately increasing the likelihood of developing truly impactful treatments that address unmet needs. For payers, they will have a more complete picture of what constitutes value to patients, assisting with difficult decisions on spending priorities. And for patients, they will be empowered and ultimately benefit from improved access to potentially transformational medicines.

For all three, the systematic integration of patient evidence into HTA/access decision-making provides an opportunity to break down silos and build long lasting relationships so at last, when patients talk, they are truly heard.

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Executive Insight is a specialized healthcare consulting firm supporting biopharmaceutical companies to successfully prepare, launch and commercialize their products.

Special contribution: **merakoi** is a social impact company providing patient expert engagement services and was a supporting partner for the patient expert survey.

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