Emphasizing the patient voice in health technology assessments

Sudesh Basra
Director
Market Access

Harrison Davis
Senior Consultant
Market Access

Sudesh Basra and Harrison Davis explore the evolving role of the patient voice in market access, and the vital role of patients in health technology assessments.

The involvement of patient voice in demonstrating the value of treatments in healthcare is gaining in importance. While gaining patient insights is not a new topic for biopharmaceutical teams, the body of literature on the role of patient centricity in health technology assessments (HTAs) is growing. As access to healthcare information has become more democratized, empowered patients are turning to social media, websites, and forums to independently seek out healthcare information. This represents an opportunity to engage patients far earlier in clinical development, gaining crucial insights and data to support the HTA process while also building lasting relationships which span the duration of the product lifecycle.

However, global progress in the involvement of patients in HTA has been challenging.[1,2] The time is ripe for pharmaceutical manufacturers to evaluate and evolve how they engage with patients, determining how and when to best incorporate patient perspectives during product development, and to discover how to use patient insights more effectively to articulate the value of their product within the HTA process.

> **Patient voices, preferences, and behaviors are crucial to determining how a product can address the clinical and disease burden, as well as unmet therapeutic needs.**
>
> *Sudesh Basra, Director, Market Access*

Patient-centric product development leads to

The benefits of patient-centric product development

The value of new health technologies is multidimensional and not limited to clinical benefit and cost. As a result, showing the nuanced benefits of a therapy requires the capture of patient value beyond traditional patient-reported outcomes.[3,4]

A patient-centric approach strengthens the value of a product across the whole development cycle. Pharmaceutical manufacturers are increasingly recognizing patients as 'consumers' because their voices, preferences, and behaviors are crucial to determining how a product can address the clinical and disease burden, as well as unmet therapeutic needs.

Patients can play an important role at multiple points in product development, including understanding how their definition of an impactful and tolerable treatment drives differentiation from the competition.

Patients are also collaborating with biopharmaceutical companies to define and improve the design of a study by reviewing the protocol and providing insights on how it impacts the burden of the disease.

Asking a patient 'What does a successful treatment look like?' uncovers issues from symptoms affecting their quality of life, to their ability to remain employed, while illuminating the patient relevance of objective endpoints, the relative importance of survival and quality-of-life gains, and issues about out-of-pocket costs. Considering 'patient-relevant' endpoints – which are outcomes that make a difference to their 'lived experience' – may drive payer value, too.

In other assessments, patient experts and advocacy groups can provide the evidence needed to confirm which outcomes are appropriate for modeling or highlight additional social and economic impacts that may inform a final decision. These impacts may be less obvious in helping to overturn a negative decision, but they are impactful nonetheless.

Additionally, these groups are more politically engaged than ever before and actively collaborating across the healthcare industry, developing extensive stakeholder networks and circles of influence. From providing insights to identifying patients and driving trial recruitment, advocacy groups can be a powerful partner for biopharmaceutical companies across the product lifecycle.

The influence of the patient voice in regulatory and health technology assessment activities

As patient perspectives are not always captured in clinical trial data, additional work is necessary to provide real-world context about the benefits and challenges of a new treatment to help decision-makers (including payers, regulators, and healthcare professionals) better evaluate its potential to address clinical and disease burden. Patient perspectives include defining which outcomes make a difference to their lived experience; for example, what level of improved symptom control or reduction in comorbidities is meaningful to patients. Understanding these patient-relevant outcomes adds more context to general patient-reported outcome measures (PROMs) such as quality-of-life assessments.

Today, patient preference data from rigorously conducted studies are providing decision-makers with a wider pool of views on the outcomes that patients value. While currently there is no formal requirement for patient preference data by either regulators or HTA agencies, the data are often considered as part of evidence reviews.

Currently, HTA agencies are recognizing the importance of systematically incorporating patient insights, but published evidence suggests that current HTA frameworks lack consideration of aspects of patient experience beyond patient-reported outcomes.[5,6]

The processes for including patient evidence lack alignment and different types of input are required from patients, patient advocates, and patient organizations in these differing processes.[7–9]

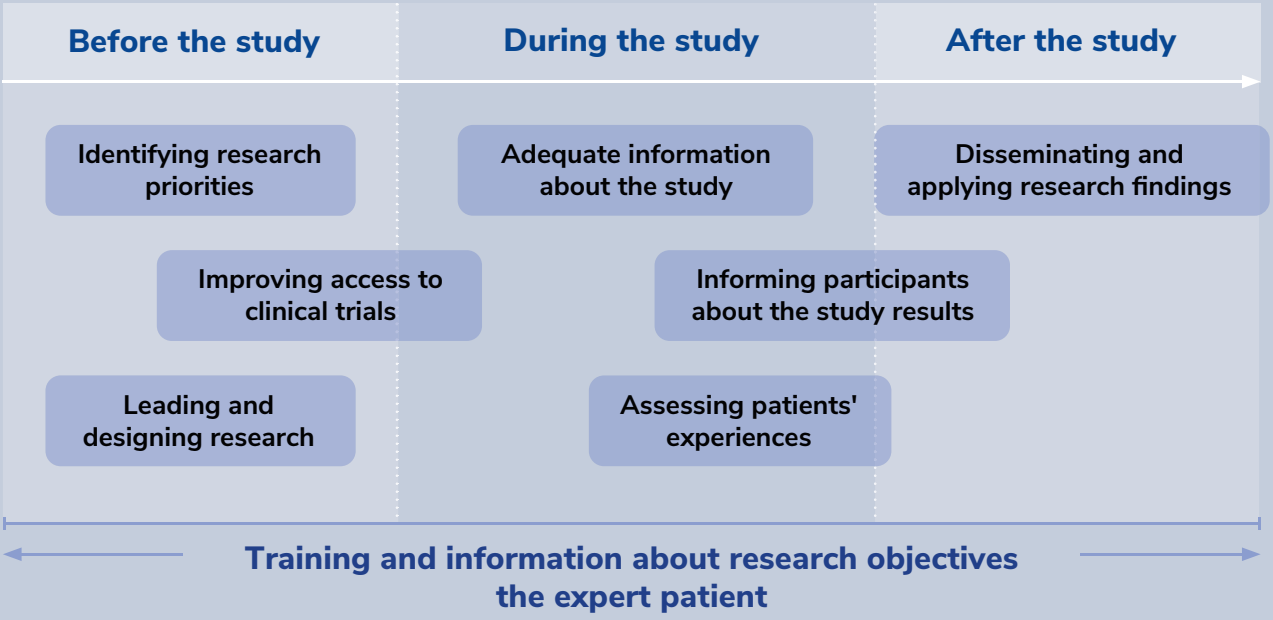
The complex nature, terminology, and evidence requirements of HTAs also make it difficult for patients to have a consistent understanding of the HTA structure, and their roles within it.[10-12] Instead, patient experts have traditionally shared their views during committee meetings or in consultation responses.

Additionally, methodological challenges such as heterogeneity of patient data, choice of analytical method, procedural issues concerned with how to evaluate the impact of patient experience studies, and adherence to evidence-based practices are slowing uptake.[11]

To combat this, the FDA has developed a new four-part series of guidance documents addressing how to systematically collect data on the patient voice, signaling the importance of more and better patient involvement.[6]

> **As health technology assessment agencies gain more applied experience of integrating patient perspectives into their review processes, manufacturers will need to come to market armed with patient insights to support their product.**

Opportunities for patient involvement across clinical development



Several HTA bodies are driving greater levels of patient engagement, including: Canadian Agency for Drug Development and Health (CADTH), Scottish Medicines Consortium (SMC), Belgium Healthcare Knowledge Centre (KCE), and Pharmaceutical Management Agency in New Zealand (PHARMAC).[12–15]

NICE, a forerunner for patient involvement, includes patients across its HTA and scientific advice work. In February 2019, NICE provided its first scientific advice on a manufacturer's patient preference study design, sending a signal to industry about the importance of these data in HTA.

Multiple external agencies have also published materials proposing how best to directly support patients who wish to become involved in HTA, including calls for proper compensation, and effective training and support.[1,16,17]

The HTAi Patient and Citizen Involvement Interest Group (PCIG) is working toward providing a comprehensive and searchable international directory of publicly available materials that have been specifically designed for patients to help them participate in HTA processes.[3]

EUPATI guidance also suggests HTA bodies should have proactive communications strategies to effectively reach, inform and enable a wide range of patients to participate fully in each HTA, including making public the criteria and decision-making processes.[1]

Recommendations include soliciting a variety of patient perspectives for HTA appraisals, both as a written statement, part of the evidence dossier, and as an oral statement during committee deliberations.[18]

These examples illustrate a growing trend toward more expert and engaged patient groups, working with both industry and HTA agencies to inform value decisions about new The HTAi Patient and Citizen Involvement Interest Group (PCIG) is working toward providing a comprehensive and searchable treatments which ultimately affect patient access to the medicines and can impact pricing decisions of payers.

Working in partnership with patients and patient groups

Patients and patient advocacy groups would benefit from support and greater education about the types of data that would be valuable input for an HTA. This support is required for patients experienced in all types of conditions, including rare diseases and disease areas for which more specialized technologies are being developed.[1,16,18]

Establishing long-term partnerships is the most likely way to successfully bring the patient voice into product development. However, effective collaboration is not always straightforward. Ethical or legal boundaries and public perception can cause concern, particularly where there is a funding relationship or new drugs under review. There are clear guidelines for industry to help overcome this, but the fear of perceived influence can still be a stumbling block.

Budgetary or organizational constraints within a manufacturer can also get in the way of partnerships with advocacy groups being prioritized. In some disease areas the scarcity of patients, competition for partnership, or a lack of established advocacy groups presents an additional challenge.

Engaging early and regularly throughout product development can help mitigate these challenges and avoid irreversible decisions being made without patient insights and input that could impact both patient outcomes and the trajectory of the launch.[1]

Additionally, collaborating on projects across the product lifecycle, beyond market access, following industry guidelines and principles, and achieving internal alignment on the importance of investing in partnership with patients can help manufacturers establish invaluable long-term partnerships with patients.

The future of patient engagement is constantly being redefined, but the direction is one of increased appreciation of the patient voice in both product development and assessment, and toward more systematic and sophisticated ways to incorporate it. This presents a challenge and an important opportunity for pharmaceutical manufacturers.

Supporting your most complex Policy, Access, Value, and Evidence needs

PRMA Consulting is a member of Fishawack Health, and together, our global Value, Evidence, and Access consultants work cross-functionally with our Policy, Medical, and Marketing teams to solve complex healthcare challenges.

Get in touch

Contact us to learn how we can help optimize access and demonstrate the full value of your innovative products to payers.

References

1. Hunter A, et al. *Front Med.* 2018;5:231.
2. Wale J, et al. *Int J Technol Assess Health Care.* 2017;33:1–4.
3. US Food & Drug Administration.
4. Nolte N, et al. *Cambridge University Press.* 2020. doi: 10.1017/9781108855464
5. Schnider ME.
6. US Food & Drug Administration.
7. Scott AM, et al. *Res Involv Engagem.* 2017:3(2).
8. Bond K, et al. *Int J Technol Assess Health Care.* 2020;36(4):445-452.
9. HTAi Patient and Citizen Involvement Group.
10. O'Rourke B, et al. *Int J Technol Assess Health Care.* 2020;36(1):1-4.
11. Rehfuess EA, et al. *J Public Health.* 2017;39(1):209-212.
12. Wale J, et al. *Int J Technol Assess Health Care.* 2020;36(3):197-203.
13. Leopold C, et al. *BMC Health Serv Res.* 2020;20(1):351.
14. Single A. Medicines N Z. 2021.
15. Cleemput I, et al.
16. Sarri G, et al. *Health Policy.* 2021;125(5):593-601.
17. HTAi.
18. Van Overbeeke E, et al. *Patient.* 2021;14(1):119-128.11.