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Patient-centered health technology assessment: a perspective on engagement in health technology assessment by three patient organizations and a health technology assessment body

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Abstract

Patient engagement in health technology assessment (HTA) has become increasingly important over the past 20 years. Academic and practitioner literature has produced numerous case studies and best practice accounts of patient involvement practices around the world. This text analyzes the experience of being involved in an Institute for Clinical and Economic Review (ICER) HTA review in the United States. The analysis comes from the joint perspective of three patient organizations: Lupus and Allied Diseases Association, Inc.; Lupus Foundation of America; and Black Women's Health Imperative, as well as ICER. We suggest that meaningful, patient-centered engagement, where patient communities are systematically integrated throughout the review, can be a way of returning to the discipline's roots focusing on technologies' societal and ethical impact. It is a process that requires robust commitment from all involved but produces assessments relevant to those directly affected by them.

Introduction

Patient engagement in health technology assessment (HTA) has been a topic of increasing importance for HTA bodies, patient organizations, and academics (1–3). Over the past two decades, HTA agencies around the world have set up processes involving patient representatives in HTA proceedings (4). Research, including a recent special issue of the *International Journal of Technology Assessment in Health Care*, has paralleled these developments to share critical analyses, case studies, and best practice accounts (5–8).

This analysis is based on the experience and perspective of the Lupus and Allied Diseases Association, Inc. (LADA), Lupus Foundation of America (LFA), Black Women's Health Imperative (BWHI), and the Institute for Clinical and Economic Review (ICER), from a lupus nephritis HTA review. We describe this collaboration offering a patient-centered review that may help policymakers understand the value treatments provide for patients and societies.

Background

In July 2020, ICER, an independent nonprofit organization producing comparative and clinical effectiveness assessments in the United States (9), decided to review two drugs for treating lupus nephritis: voclosporin (a new drug seeking Food and Drug Administration [FDA] approval) and belimumab (a drug approved in 2011 to treat systemic lupus erythematosus, seeking an expanded indication).

Fifty percent of people living with systemic lupus erythematosus which disproportionally affects women, will go on to develop lupus nephritis. Particularly those younger in age, male or of black, Hispanic, or Asian origin (10). Lupus nephritis is characterized by inflammation and progressive damage to the kidneys which can lead to renal failure. Given the relatively high numbers for lupus nephritis in the United States (20 per 100,000 people) (11), the drugs being reviewed were associated with a higher budget impact than existing treatments.

In August 2020, ICER opened the lupus nephritis HTA review and invited input on the scope from several patient organizations, including LADA (12) and LFA (13), later joined by BWHI (14). The lupus nephritis review was the first ICER assessment carried out in its entirety following ICER's enhanced and more systematic patient engagement program, part of its 2020 Value Assessment

Framework (15;16). It was also the first time ICER extended its outreach beyond national policy- and research-focused organizations, such as BWHI (which had contributed to previous ICER reviews), to include all-volunteer patient-led organizations such as LADA and LFA. In its final report published in April 2021, ICER found both therapies to have benefits for patients and to be cost-effective (17).

Collaborating in practice

ICER's review process for the lupus nephritis drugs lasted 8 months. ICER identified and invited relevant patient organizations before starting its scoping process; other organizations could join later. ICER sought input at each of the five key stages (scoping; modeling analysis plan; draft evidence report; preparation for the appraisal meeting; and, for the first time, policy recommendations). Patient organizations provided public written comments and oral feedback according to their distinctive perspectives – patient, social, and policy – on ICER's draft scoping document and modeling analysis plan, as well as on the draft evidence report. They also participated in around five video calls with ICER's stakeholder engagement, clinical research, and economic modeling teams.

LADA's President provided an expert review of the draft evidence report and policy recommendations. She, a second patient expert and BWHI's President took part in the policy roundtable, which produced recommendations on the reviewed drugs. Representatives from LADA and LFA also spoke during the March 2021 public meeting of the New England Comparative Effectiveness Public Advisory Council (CEPAC), ICER's regional HTA appraisal committee (9).

For the purposes of writing this piece, the authors met for two video calls to discuss and compare their perspectives on the process. They also submitted further written detail of their approach and reflections.

Challenges during the collaboration

The review process required important commitment from the patient organizations in terms of human resources, prioritization, and strategy. ICER aims to publish its reviews to coincide with the health technology's marketing authorization (9), making assessment timelines short. Although timely assessments are sought after by payers (18), the lupus nephritis review demonstrated that patient organizations must quickly reallocate resources to meet deadlines. For BWHI, this was six staff members and consultants; for LADA, eight volunteers; for LFA, fourteen staff members, external expert advisers, and consultants. All three organizations had to draw on multiple team members to provide the needed diverse and specialist expertise. As an example, BWHI's team consisted of its President, a data scientist, the Chief Policy Officer, a junior policy officer, and two economists. On ICER's side, two full-time staff worked directly with the organizations throughout the review as needed.

Familiarity with HTA and ICER's processes varied within the organizations' teams. Education of internal and external partners about the purpose of participating in the review required time and attention. This was partly due to the complexity of HTA as applied, policy-oriented research; and partly due to ICER's specific role within the United States health system. As a private nonprofit organization, ICER's reports and policy recommendations are nonbinding but nonetheless influential with policymakers (19;20).

Although ICER's methodology and consideration of cost would be standard in most countries with public HTA institutions, it is less known and accepted in the United States. As a result, during the lupus nephritis review the patient organizations needed to provide information to their communities and external partners about ICER's mission, as well as broader issues around pricing, coverage, and crucially, the importance of having their voices represented in the HTA process. Their attention did bring benefit. LADA and LFA, for example, found that bringing together the organizations' leadership to understand the purpose of the review and their participation enabled the organization to be actively engaged at each stage of the review and after its final publication.

In practice, the speed of the review, the need for diverse experts, and various levels of familiarity with the HTA process meant that patient organizations needed to rapidly rearrange their priorities. This urgency was a challenge even for larger organizations. However, LADA's active participation showed that with commitment to prioritize the review and a clear strategy for getting their main goals included, even smaller, patient-led, all-volunteer organizations can effectively represent their communities.

In recognition of this commitment, while ICER does not provide compensation for the overall involvement of the patient community in their assessment processes, they did provide standardized honoraria to individual expert patients involved in specific activities. These activities included the review of the draft evidence report, the public meeting, and policy roundtable. Compensation was standardized per expert and activity, aligned to National Health Council recommendations (21;22).

Value of the collaboration

For the patient organizations, participating in the HTA process brought an opportunity to shape the scope, parameters, and context of the assessment, particularly through ICER's early involvement of patients and other stakeholders during scoping. LADA, LFA, and BWHI joined ICER's review to put forward the needs and priorities of people with lupus nephritis and their caregivers and to present what constitutes value from the patient perspective. Ultimately, they influenced the weight given to their priorities in health economic modeling and appraisal deliberations.

For example, LADA and BWHI underlined the importance of considering patients' and caregivers' career, educational, and family life goals, particularly motherhood (often difficult to achieve on immunomodulating drugs that can be teratogenic), in addition to existing clinical and economic aspects. ICER included these factors as "contextual considerations" and "potential other benefits" in its report. Perhaps most importantly, after discussions with LADA, ICER modified the questions on which the appraisal committee voted, which formed the basis of its policy recommendations, to include disease-specific contextual considerations.

For ICER, collaboration with LADA, LFA, and BWHI brought expert knowledge of the disease and standards of care in practice. The groups' early involvement helped ICER identify gaps in available data and literature, and potential sources of additional data. For instance, LADA, LFA, and Lupus Research Alliance's 2017 survey of lupus patients and caregivers (23) which was aligned to the FDA's Center for Drug Evaluation and Research (CDER) Patient-Focused Drug Development program (24). This survey, comprising new, patient-generated quantitative and qualitative data, was a valuable source of what Staniszewska and Söderholm Werkö call "patient-based evidence" (25) for the final report, which notably underscored the importance of contextual considerations.

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The groups also noted gaps that ICER addressed in the review or during the appraisal stage. For example, despite the disproportionate number of lupus nephritis patients coming from communities of color, clinical trial efficacy data for subpopulations for the two drugs by race/ethnicity was limited. Based on discussions with the patient groups, ICER included a scenario analysis for Black patients in its assessment, as advocated for by BWHI. Here ICER noted the report's limitations given insufficient trial data and used the occasion to emphasize the need for pharmaceutical developers to include patients from populations affected by the disease in clinical trials. The authors do recognize that beyond the efforts, in this case, opportunities exist to incorporate even more patient experience data, in line with efforts led by the United States' patient community, using and referencing the agenda-setting work by the European IMI-PREFER Consortium project (26).

As expected by the concepts of knowledge transfer and coproduction (27;28), the value of the collaboration continued after the review's publication. All three organizations (plus ICER) disseminated the results to their communities and external stakeholders. LADA and LFA conducted outreach to healthcare payers, including sending letters to encourage the placement of both drugs on formularies without burdensome utilization management policies. The groups also used the report in their engagement with policymakers, including advocates within the United States Congress. For BWHI the report was an opportunity to continue its work with legislators, particularly the Congressional Black Caucus, on coverage decision making and issues of value more broadly.

Similarly, for ICER, understanding the lived experience from diverse patient voices and methodological choices informed by discussions with patient groups (for instance, ICER's decision to avoid using ethnicity-specific cost and utility values to limit reinforcing existing health inequalities), are likely to have impacts beyond the two drugs assessed. It sets a new standard of evaluation not only for future lupus nephritis treatments, but also for other disease areas with significant racial disparities or underserved and understudied populations.

Discussion

This was ICER's first application of their enhanced, systematic patient engagement approach, which has been embedded into their value assessment framework (15). This provided an opportunity to assess the methodology and share learnings more broadly.

Meaningful patient engagement can be a way of going back to "full HTA" and the discipline's roots, focusing on technologies' societal and ethical impact (25). Regardless of the HTA outcome, patient engagement is a demanding but critical process for both patient organizations and HTA bodies, especially if patient communities are engaged at all stages of the assessment.

For patients, they know even a favorable HTA decision does not guarantee access but being involved and heard is crucial, regardless of the outcome. Collaboration enables consideration of their priorities. A positive HTA review can be used as evidence-based argumentation for advocacy with payers, clinicians, and decision makers. An HTA review is an occasion to engage on topics of clinical trial design, regulatory clarity, pricing, coverage, and access with patient groups' members and external partners, regardless of the outcome. This can have long-term benefits, especially in patient communities that are unfamiliar with the details of access issues. An HTA report can also be a reminder of a patient community's unmet needs and gaps in evidence.

For HTA bodies, patient groups bring detailed knowledge of the disease and standards of care in practice; familiarity with patient, clinician, and policymaker communities; and an understanding of the priorities and needs of patients and their caregivers, especially when these are ill-captured by traditional clinical and economic factors. In line with our experience, HTA bodies and regulators express interest in incorporating patient preferences in their decisions, although they do not give them the same weighting as health economic evidence (29–31). Recent, rapidly growing literature on patient preferences by IMI-PREFER (26;29) provides advice on identifying patient preferences through qualitative and quantitative methods (32;33). As suggested by our case study, systematic collaboration between HTA bodies and patient organizations can make going beyond these traditional factors easier to operationalize, prioritize and include in decision making in a meaningful way.

Although this was a single review in a single country, we believe practical learnings can be drawn from our experience:

1. Patient organizations and HTA bodies should be aware that participating in HTA processes requires commitment but can bring substantial benefits.
2. Patient organizations should clearly define their goals and reasons for participating in the assessment from the outset. These should be communicated to their members, leadership, and external partners.
3. Patient organizations should recognize they have the expertise to contribute to HTA. They are experts on the lived experience, including the patient and societal factors that influence perception of value. Specialist expertise (e.g., in health economics) is not a prerequisite for participation.
4. Patient organizations should draw on their community members to recommend the most appropriate patient and physician representatives to provide diverse perspectives.
5. Patient organizations should expect to follow up with payers, policymakers, and clinicians around HTA reports, irrespective of the results. Even positive HTA recommendations may require outreach to improve access.
6. For HTA bodies, assessments can be greatly enhanced by having multiple patient organizations provide diverse perspectives.
7. HTA bodies can support patient participation by providing clear instructions and materials and encouraging communication.
8. HTA bodies and patient organizations should continue to work together to find meaningful ways to enhance collection of qualitative patient data and its use within HTA, aligned with recommendations from the FDA's Patient-Focused Drug Development program to ensure patients preferences are captured (26;29).
9. Learning from organizations already familiar with an HTA process may be useful for patient organizations engaged in HTA for the first time.

Conclusions

When HTA bodies and patient organizations demonstrate a strong commitment to partnership, patient engagement becomes an integral part of the HTA process.

Patient-centered HTA integrates patient communities throughout the entire review process. This approach takes commitment but produces assessments relevant to those directly affected by them, which may lead to improved access for the patient community.

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