The Impact of Patient-Centered Engagement on ICER Drug Reviews

Background
In the United States, decisions around drug pricing and access are often made without patients. The Institute for Clinical and Economic Review (ICER) hopes to change this by inviting the patient community to participate in our evaluation of how well new drugs work and what a fair price should be. This allows the patient community to make sure the patient voice is included in decisions that impact their health, finances, and access to a new treatment.

Objectives
- To invite and incorporate patient community input into ICER’s evaluation of new drugs for lupus nephritis (LN), hemophilia, and metachromatic leukodystrophy (MLD)
- To elevate patient and caregiver insights to inform decisions about drug pricing and insurance coverage in the US

Participants
Across ICER’s three reviews on LN, hemophilia, and MLD, total participation from patient groups and individual patients/caregivers was as follows:

- 9 patient groups
- 7 panelists at public meeting
- 8 oral commenters at public meeting
- 9 participants for small-group interviews

Patient Insights to Impact

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<th>ICER Review</th>
<th>What ICER Learned</th>
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<td>Lupus Nephritis</td>
<td>1. Huge negative impact on patients’ ability to work, to have children, and to advance in their careers. 2. Disparities in health outcomes among communities of color</td>
<td>1. ICER’s LN public meeting addressed patients’ concerns about future fertility. 2. ICER conducted analyses to explore disparities among Black versus white LN patients.</td>
<td>Economic &amp; Policy</td>
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<td>Hemophilia</td>
<td>1. Increased quality of life with one-time gene therapy by reducing lifetime treatment burden. 2. Financial burden associated with current high-cost hemophilia treatments.</td>
<td>1. The hemophilia quality of life data increased ICER’s value-based price for the gene therapies under review. 2. ICER’s hemophilia policy recommendation called on the government to step in and ensure more reasonable pricing.</td>
<td>Economic &amp; Policy</td>
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<td>Metachromatic Leukodystrophy</td>
<td>1. Data to support improved patient benefit with treatment for a subpopulation. 2. Transportation &amp; housing costs are barriers when needing to travel to receive gene therapy.</td>
<td>1. ICER adjusted the clinical rating for the MLD gene therapy to better reflect its increased patient benefit. 2. ICER’s MLD policy recommendation included wraparound coverage for transportation &amp; housing costs.</td>
<td>Clinical &amp; Policy</td>
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Project Design
The ICER review process typically lasts nine months and actively involves the patient community from start to finish. The figure below illustrates the flow from phase to phase, and the range of opportunities ICER offers for patient community input. For ICER’s review of lupus nephritis (2021), hemophilia (2022), and metachromatic leukodystrophy (2023), the key patient advocacy groups as well as individual patients/caregivers were invited to participate in the opportunities outlined below.

Key Takeaways
- Patients and caregivers are the experts in the lived experience and can provide meaningful insights beyond what may be captured in clinical trials and other published data.
- Although participating in the ICER process can require time and staff resources, this investment allows the patient community to educate policy makers about their lived experience and inform decisions about drug pricing and insurance coverage.
- ICER’s reports are often reviewed by insurance plans who do not otherwise have information about the patient lived experience when making decisions about drug coverage.

Conclusion
The impact of patient engagement as reflected by these three case studies reveals how patient preferences, patient-reported outcomes, and input on lived experiences can shape ICER’s report conclusions and recommendations.

View An ICER Snapshot
Use the QR code to view ICER’s Snapshot (a patient-friendly report summary) for the MLD review. This summary includes key clinical and economic results as well as how patient community input impacted ICER’s analyses and policy recommendations.

Participating in the ICER process is one way patients can have a voice in how fair pricing and fair access is determined for new treatments.