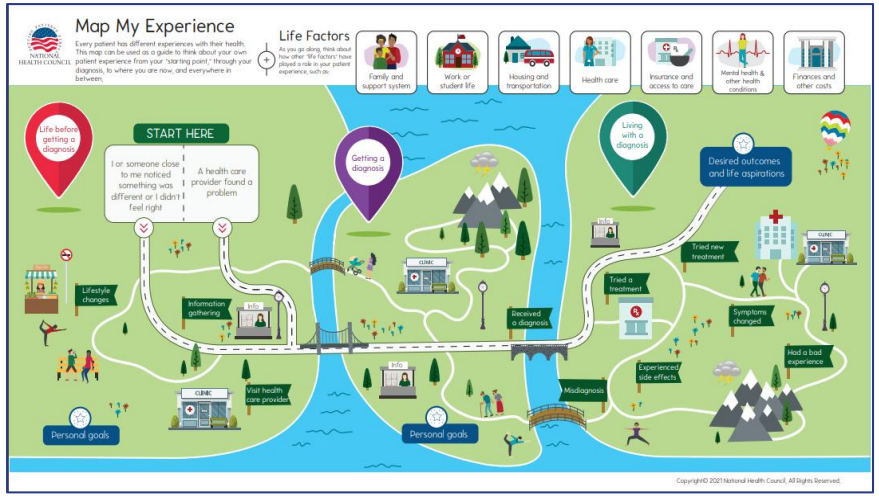
We know how to engage patients.



We know what data patients can provide.

EMA

Reflection Paper EMA/CHMP/PRAC/148869/2025 (Draft, Sept 2025)

"...data that directly reflect the experience of a patient or carer, **without input or interpretation** by a healthcare professional, third party or (artificial intelligence [AI]-based) device."

FDA

21st Century Cures Act (2016) amended by FDARA (2017)

"...data that (1) are **collected by any persons**...; and (2) are intended to provide information about patients' experiences with a disease or condition, including (A) the impact (including physical and psychosocial impacts) of such disease or condition or a related therapy or clinical investigation; and (B) patient preferences with respect to treatment of the disease or condition."

We agree that patients should be engaged in value and health technology assessment.

Table 3. Agreement on recommendations after round 2 (n = 20).

Topic	Recommendations	Agree or Strongly Agree percentage
Patient and caregiver engagement	Patient input should be gathered where relevant throughout the planning, conduct, and validation of a V/HTA to maximize its impact.	100.00%
	V/HTA entities should allocate resources to train and support patients and patient organizations to collaborate in V/HTA (eg, scoping, interpretation).	80.00%
	Presentations, drafts, and other materials on which patients and patient communities are asked to comment should be in plain, accessible language.	95.00%
	To encourage financial diversity among patients contributing their time to assist with data-gathering efforts and other contributions throughout a V/HTA (eg, interpretation, scoping), the entity commissioning a V/HTA (eg, V/HTA body) should provide funding or help patients and patient organizations secure funding.	70.00%*
	Recognizing the large share of healthcare paid for by employers, an effort should be made to bring patients and employers together to provide input, particularly in the creation and application of benefits.	80.00%

Source: Slejko, Julia F., et al. "Achieving Patient-Centered Value/Health Technology Assessment: Recommendations From a Multistakeholder eDelphi Panel." Value in Health (2025).

We agree that the patient perspective should be included in assessing assumptions and limitations.

populations (eg, ethnicity, disease severity, length of time living with a condition) and experiences, and outcomes important to patients.	
Economic evaluation should include all relevant costs (opportunity, indirect, out-of-pocket medical, and non-medical) incurred by patients and their caregivers.	95.00%
The availability of relevant data and evidence (eg, randomized controlled trials, real-world data/ evidence) to support a V/HTA and economic evaluations should guide the framing, scope, and timing of initial V/HTAs and updates.	85.00%
Assumptions and limitations of an economic evaluation should be clearly and systematically articulated in the report, along with any related biases in the results.	95.00%
Patients and patient communities should be able to submit their perspectives on the impact of the assumptions and limitations through mechanisms such as a public comment period.	100.00%
If patient organizations are collecting data for an assessment, this should be done in collaboration with a researcher, either internal or external, so that the data developed is fit for purpose. If researchers are collecting data for an assessment, patient organizations should be given the opportunity to collaborate.	90.00%
Data collected by patient organizations may represent the best available data and evidence. In these cases, these data or evidence should be integrated into models or guide model development. They should be presented alongside appropriate limitations, assumptions, and caveats as required for any analysis input.	80.00%

Source: Slejko, Julia F., et al. "Achieving Patient-Centered Value/Health Technology Assessment: Recommendations From a Multistakeholder eDelphi Panel." Value in Health (2025).

For debate:

How should patient-identified outcomes be incorporated when they fall outside traditional cost-per-QALY frameworks?

When does lived experience justify revisiting structural assumptions?

How might patient-informed perspectives influence cost-effectiveness results, equity considerations, and uncertainty analysis?

What safeguards are needed to prevent bias, double-counting, or unintended scope expansion?