

THE IMPACT of Patient Involvement in HTA

*Overcoming Barriers with
Practical Solutions*



Annual
Conference
2025

Natacha Bolanos | Patient Representative

**LYMPHOMA
COALITION** 

Seeing through the lens of the patients



WHY PATIENT INVOLVEMENT MATTERS IN HTA

Beyond Clinical & Economic Data:

- Patients offer real-world insights on tolerability, psychological impact, and quality of life, ensuring decisions reflect lived experiences.

Identifying True Innovation:

- Patient input highlights unmet needs and benefits beyond clinical endpoints, aligning treatments with real patient priorities and healthcare demands.



CURRENT STATE OF PATIENT INVOLVEMENT

GOOD PRACTICES

- Some agencies (e.g., NICE, CAD, SMC) have established frameworks for patient inclusion.
- Others also have specific guidelines (CDA)
- Use of qualitative data to complement clinical evidence (e.g., antimicrobial wound dressings in Scotland).

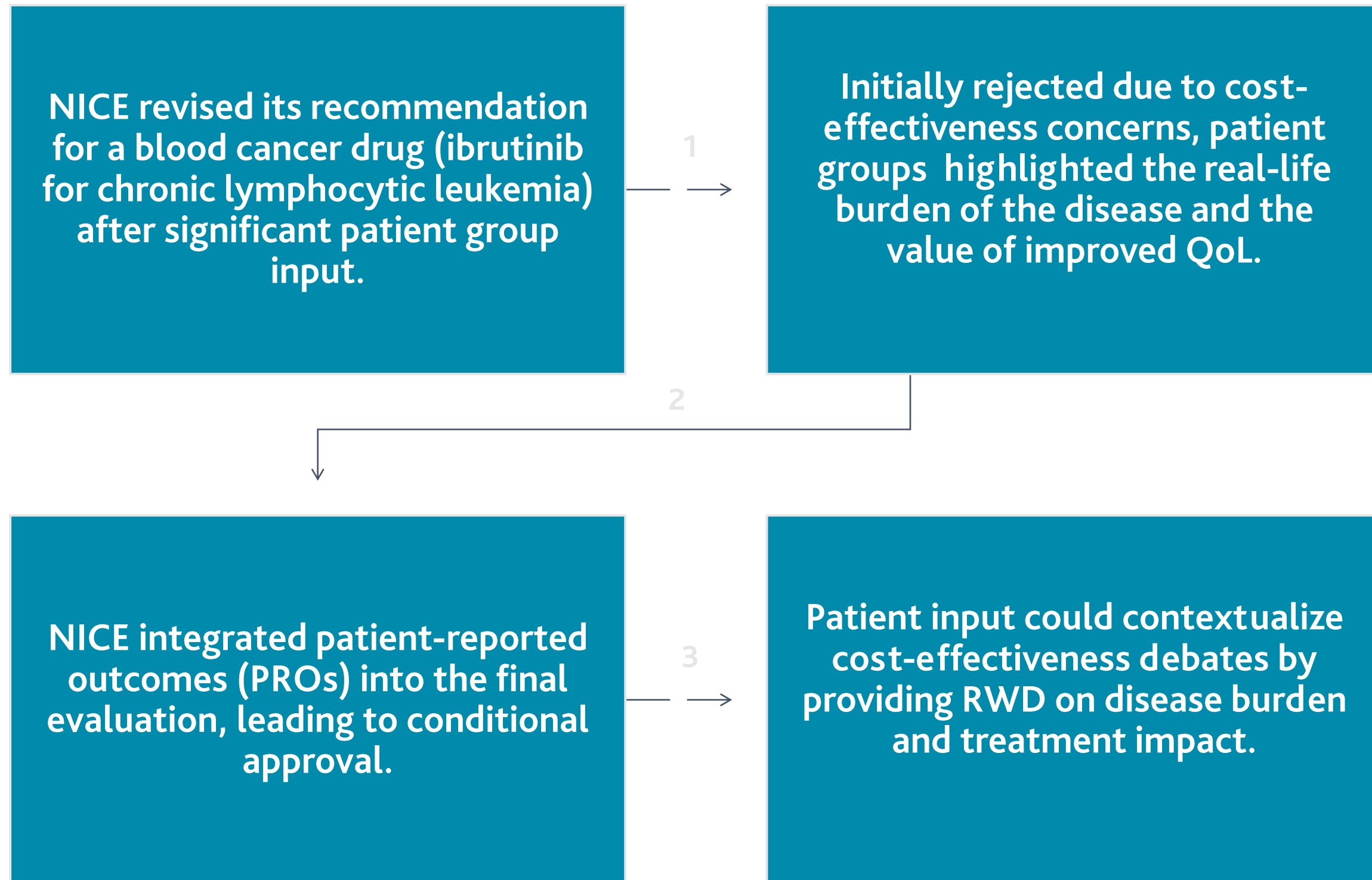
CHALLENGES

- Lack of consistency in patient involvement across Europe.
- Patients often lack the necessary knowledge/training to engage effectively.
- Tokenistic involvement vs. meaningful participation



CASE STUDY: NICE (UK) – BLOOD CANCER DRUG ACCESS

● **Early and structured patient involvement can lead to decision reversals.**



CASE STUDY: CADTH (CANADA) – Oncology Drug Review for Multiple Myeloma

● Direct patient engagement enhances the relevance of clinical and economic assessments

The pan-Canadian Oncology Drug Review (pCODR) engaged multiple myeloma patient groups during an HTA for a novel immunotherapy. Patients provided insights on treatment side effects, disease progression, and caregiver burden. Patient submissions led to an expanded interpretation of clinical trial data, emphasizing symptom relief and long-term survivorship goals.



HTA committee members acknowledged the role of patient perspectives in adjusting health economic models to better reflect real-world experiences.

BARRIERS TO MEANINGFUL Patient Involvement

Lack of Institutional Memory:

Experts rotate out, experiential knowledge may be lost.

Stakeholder Reluctance:

Concerns about perceived bias and lack of “objective” data.

Resource Constraints:

Lack of funding and training opportunities limit full engagement.

Process Complexity:

HTA methodologies are often not designed with patient accessibility in mind.



SCARCE DATA ON Patient-Reported Outcomes



NOT
EVIDENCE



PROMs in Lymphoma Clinical Trials - 2009-2020

From 1541 identified clinical trials
129 reported PROM use (8%!)

The most used PROMs:

- FACT-Lym,
- EORTC QLQ-C30
- EQ-5D



HTA Reports in lymphoma (NHL) 2015-2020

Accepted as relevant PROMs for
lymphoma

- FACT-Lym
- 36-Item Short Form Health
Survey (SF-36)
- Skindex-29 were

Treatment benefit reported by PRO
data was limited

Patient Experience Data for HTA Submissions

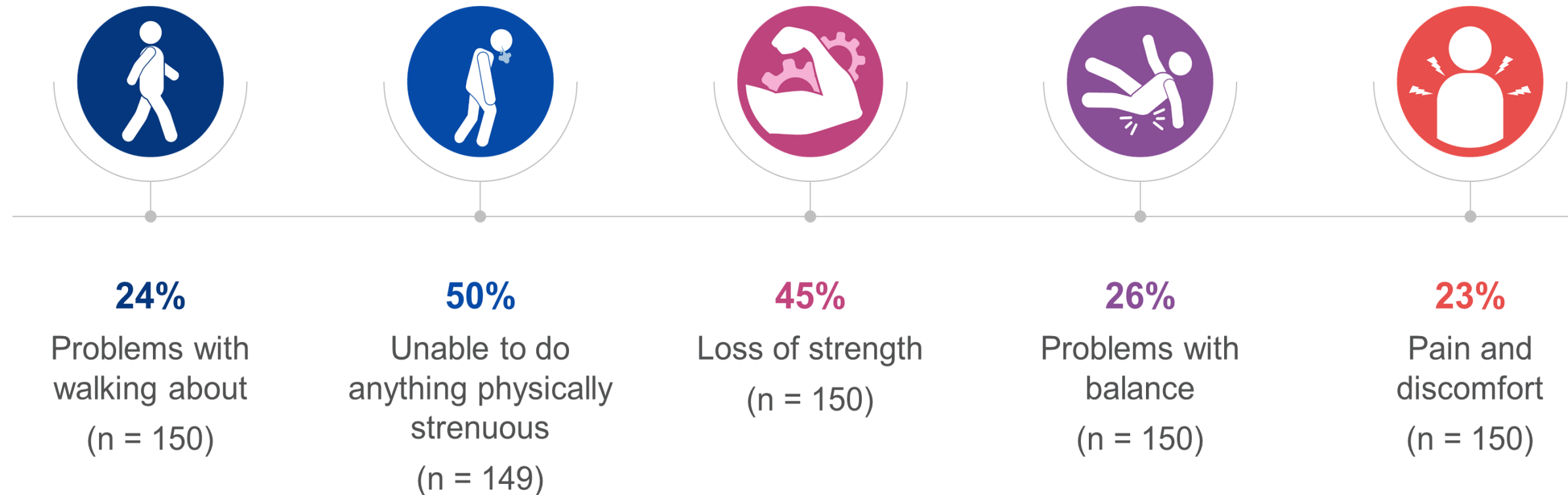
Global Patient Survey (GPS) on Lymphomas and CLL



Global Patient Survey (GPS) on Lymphomas & CLL

- Biennial online global survey
- Live February–April 2024 in 20 languages
- 2024 overall number of completed responses 11,170 from 79 countries including:
 - 9,693 patients (87%)
 - 1,477 caregivers (13%)

Example of findings: Quality of Life in R/R DLBCL



Patient Experience Data for HTA Submissions



Patient Advocate Input to HTA / Therapy Approval Processes

The following is a synopsis of data from the GPS that patient advocates can bring forward in an HTA or therapy approval process. Outline of information required is taken from the Pan Canadian Oncology Drug Review Process (pCODR) and the National Institute for Health and Care Excellence (NICE).

CLL Relapsed Refractory 2+ times

LC 2022 Global Patient Survey on Lymphomas & CLL

1. Demographics

- CLL, relapsed/refractory 2 or more times, n=113 patients
- 48% male, 52% female
- Age distribution: 1% 18-34 years, 8% 35-45 years, 33% 46-64 years, 58% age 65+
- 19% from Asia-Pacific countries, 69% from Europe, 11% from North America
- 42% live only with a partner or spouse, 36% live with a partner or spouse and children, 17% are single and live alone, 4% are single but live with others, 1% did not say
- 23% currently employed, 64% retired, 6% unemployed, most of whom cannot work due to health reasons, 4% are homemakers, 4% did not provide details
- 1% diagnosed between 2020-2022 (time of survey), 9% diagnosed between 2017-2020, 90% diagnosed before 2017

2. Impact of the Disease

Symptoms experienced by respondents (95 patients answered this question). Five most common are highlighted.

21%	Abdominal swelling
43%	Anaemia
34%	Bone pain
53%	Easily bruised or bleed
68%	Enlarged lymph nodes / Abnormal painless swelling(s) on the body
80%	Fatigue
54%	Fever, chills, night sweats and weight loss (B-symptoms)
53%	Frequent or repeated infections
26%	Headaches
42%	Itchy skin
18%	Pain
40%	Shortness of breath
34%	Skin rashes / lesions
7%	Other
2%	No symptoms



Patient Advocate Input to HTA / Therapy Approval Processes

74% of patients (n=104) reported psychosocial issues in the past 12 months due to their lymphoma. The most reported issues are fear of progression (42%), fear of relapse (28%), isolation (26%), anxiety (24%), and depression (21%).

For those experiencing lymphoma-related fatigue, 69% say it impacts their general activity, 66% can no longer do the same physical activities, 55% say it impacts their ability to do general work and chores around the house, 44% say it affects their mood, 44% say it affects their enjoyment of life (n=77). Patients rely on balancing their time schedules – planning their activities at times of the day when they have the most energy – to cope (true for 36% of patients, n=75). 33% have also introduced exercise programs to help them cope.

60% say their immediate family understands the chronic nature of their cancer and support them, but only 34% of their extended family and friends have the same understanding and support.

3. Experiences with Currently Available Treatments

- 12% of respondents have had treatment and are in active monitoring (watch and wait)
- 67% are currently receiving treatment
- 7% finished treatment and are on maintenance therapy
- 3% in remission
- 6% finished or stopped treatment but not in remission
- 4% relapsed or refractory but not currently in treatment.

11% have had one line of therapy, 15% had 2 lines of therapy, 36% had 3 lines of therapy, 10% had 4 lines of therapy, 9% had 5 lines of therapy, and 6% had 6 or more lines of therapy. 12% are not sure or don't remember.

90% received treatment within the last 2 years.

Patients had the following treatments (94 patients answered this question):

9%	Autologous or allogeneic stem cell transplant (bone marrow transplant)
54%	Chemo-immunotherapy (e.g., R-CHOP, BR-Bendamustine rituximab)
32%	Chemotherapy alone (e.g., CHOP, chlorambucil, Bendamustine, ABVD)
1%	Chimeric antigen receptor T cell therapy (CAR-T)
3%	Complementary and alternative medicine (CAM) (e.g., acupuncture, supplements)
24%	Immunotherapy only
6%	Radiation therapy
12%	Skin creams and ointments
16%	Steroids
43%	Targeted therapy



Patient Advocate Input to HTA / Therapy Approval Processes

- 3% Topical steroid creams
- 19% Other

The majority received chemoimmunotherapy (54%), which is delivered in a hospital but can be in the outpatient clinic. 43% have been treated with a new targeted therapy, which are mainly taken at home.

43% had barriers to accessing treatment (n=106). The top ranked barriers included 19% had financial difficulties, 18% said they had difficulties accessing the most up-to-date treatments, 15% had to travel outside of the area they live for treatment, 13% mentioned treatment waiting times, 13% spoke of issues accessing a specialist, and 13% said travel to the treatment centre was burdensome.

Patients were asked if the treatment relieved their symptoms. 95 answered. Treatment was most likely to resolve enlarged lymph nodes. Fatigue, the most reported symptom, was resolved in about two out of every five patients.

53%	Abdominal swelling
73%	Anaemia
33%	Bone pain
31%	Easily bruised or bleed
89%	Enlarged lymph nodes / Abnormal painless swelling(s) on the body
39%	Fatigue
73%	Fever, chills, night sweats and weight loss (B-symptoms)
49%	Frequent or repeated infections
32%	Headaches
36%	Itchy skin
24%	Pain
35%	Shortness of breath
47%	Skin rashes / lesions
57%	Other

Patients experienced the following side effects from treatment. The top 5 are highlighted.

37%	Anaemia
20%	Cardiovascular (heart) problems
26%	Constipation
3%	Cytokine release syndrome (CRS)
32%	Changes in sleep patterns (i.e., trouble sleeping, etc.)
22%	Changes in taste and smell
43%	Diarrhoea
2%	Deep vein thrombosis

UNLOCKING THE FULL POTENTIAL OF PATIENT INVOLVEMENT

A strategic Framework to Moving Beyond Tokenism to Impactful Integration for Action

Strategic Positioning of Patient Contributions:

- Embed patients at critical decision points (e.g., early scoping, economic modeling, PICO, and post-recommendation phases), and make clear communication on how patient input is used in decision-making.
- Advocate for designated patient liaison roles within HTA bodies to facilitate direct contribution.

Embedding Patient Data as a Decision Catalyst:

- Fit for purpose data collection: Ensure patient experience data (PED) informs cost-effectiveness thresholds and value frameworks. Encouraging the use of patient experience data in value assessments.
- Push for the inclusion of patient-defined success metrics alongside clinical endpoints.

Institutional Accountability for Patient-Centered Decisions:

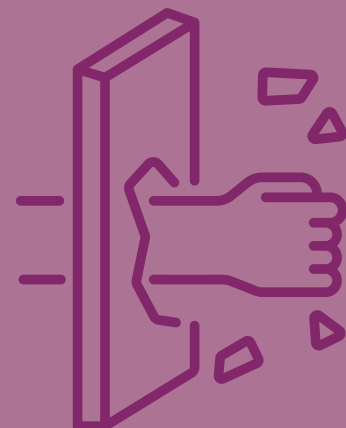
- Establish KPIs to track and report how patient input influences HTA recommendations.
- Advocate for mandated reporting and transparency on patient impact within final HTA reports and policy updates.

BREAKING Systemic Barriers

Regulatory & Policy Alignment:

Leverage the upcoming EU HTA Regulation to push for a unified, mandatory framework for patient involvement across all national HTA bodies.

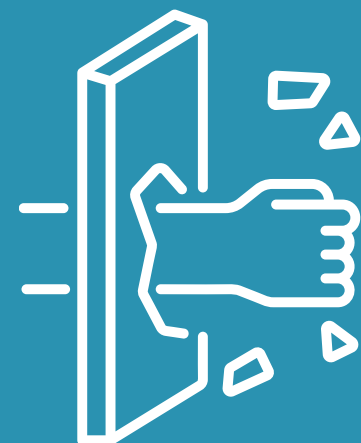
Collaborate with regulatory alliances to create a white paper demonstrating the economic and clinical value of PED.



Elevating Patient Expertise in HTA Committees:

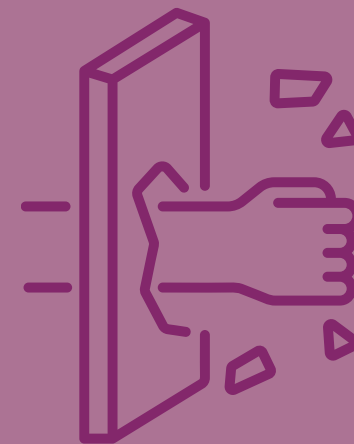
Push for policy changes to secure permanent seats for patient representatives with voting influence.

Present success cases where patient experts influenced decision reversals based on real-world burden insights.



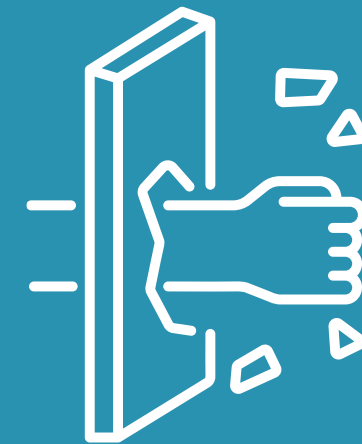
Economic Justification of Patient Engagement:

Develop economic models demonstrating how patient-informed decisions reduce post-HTA appeals and improve cost-effectiveness estimates to shift perception of patient involvement from a cost driver, to a value enabler.



Capacity Building Beyond Education:

Develop specialized training on influencing health economics, evidence interpretation, and policy advocacy.



A BOLD CALL TO ACTION



Achieve Systemic Integration:

Push for the formalization of "Patient Experience Panels" within HTA workflows, with predefined roles and impact assessment mechanisms.



Establish Economic Viability of Patient Inclusion:

Propose a sustainable funding model where HTA agencies co-fund patient group participation in assessments.



Develop a Harmonized EU-Level Patient Engagement Framework:

Engage policymakers, HTA agencies, and patient groups to ensure the 2025 EU HTA Regulation embeds robust patient engagement standards.

**THANK
YOU!**

natachab@lymphomacoalition.org

lymphomacoalition.org

**LYMPHOMA
COALITION** 