Do Patient Reported Outcomes Impact Payer Decisions? A Qualitative Study

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Background

In recent years, the role of the patient voice in regulatory and Health Technology Assessment (HTA) decision-making has grown considerably[1]. Despite efforts to include Patient-Reported Outcomes (PRO) in clinical trials to capture patient experiences, these insights are frequently overlooked in HTA discussions, particularly in oncology[2,3]. Payers also have varying requirements for how PRO data should be collected, measured, and analyzed, leading to inconsistent interpretations of results[4]. Moreover, the tools commonly used in oncology to assess patient outcomes, such as the QLQ-C30, EQ-5D, SF-36, BPI-sf, and QLQ-BR23, have seen little innovation. Patient communities have raised concerns about outdated measures and the oversimplification of outcomes. They want greater transparency on how their input is used and the extent of its impact on assessments and decisions[5]. However, without clear evidence of how PRO data influences decision-making, there is a reluctance to drive further development of new PROs in oncology.

Objective

The primary research objective was to understand the impact of the patient voice and PRO measures in access and reimbursement decisions for oncology drug development. Supportive objectives were:

- Identify current priorities of the patient voice and PRO measures within payers' decision-making process for access and reimbursement.
- Determine the types of preferred patient-reported data, data sources, and additional data types of value to payers.
- Ascertain future opportunities for the industry to utilize PRO measures.

Methods

The study included 60-minute qualitative interviews with 6 payers from US, Canada, and German (two each).

Payer participants were selected using inclusion/exclusion criteria that was applied through an online screening survey. A screener survey questionnaire was distributed across a sample of payers in the United States, Canada, and Germany. The survey included questions that gauged the knowledge around current and future policies regarding cancer treatments to determine eligibility to participate in the study.

Selected payers were then scheduled to participate in a telephone interview. A semi-structured interview guide was used to discuss the following topics:

- Priority of Patient Voice and Breast Cancer
- Patient Reported Outcomes: Measurement, Instruments, and Data
- Future Opportunities and Recommendations

Responses during the interviews were annotated and captured in a structured table. The data collected were thematically analyzed.

Results

Payer Respondent Characteristics

- All conducted access/reimbursement reviews in solid tumor oncology (e.g. breast and lung)
- Involved in formulary access, guidelines, and quality assurance schemes
  - US: National health plan
  - German: Advisors for drug reimbursement and statutory health insurance (SHI);
  - Canada: Advisors in the Canadian Agency for Drugs and Technologies in Health (CADTH)

Priority of Patient Voice and Breast Cancer

- The patient voice impact on access and reimbursement decisions differed across countries: whereby payers in the US indicated lower impact, compared to moderate impact with Canadian payers, and a higher impact with German payers.
- Patient advocacy groups exhibit less influence in the US and Canada unlike Germany, where their role on the Gemeinsamer Bundesausschuss (GBA) committee is more prominent.
- Management of BC is not prioritized over other cancers across all three countries, however overall oncology is top of mind for payers due to the population size, high-cost treatments, and significant number of new treatments entering the market

Patient Reported Outcomes

- Randomized clinical trials (RCTs) are the most preferred method of PRO measures data collection compared with real-world evidence, peer-reviewed studies, and information or research from advocacy groups.
- Payers in the US and Canada were mixed on the need to develop new PRO measures that could improve the collection of additional information, however German payers are satisfied with current measures.
- In the US or Canada, impact of additional PROs on policy is not significant whereas Germany has experienced some policy change including a wider range for BC screening based on outcomes presented.
- In terms of additional PRO evidence to secure positive access, no major changes are expected in the next three to five years. However, payers noted that quality of life measures could be used as a differentiator in a heavily competitive market where traditional efficacy measures become less distinguished.

Future Opportunities and Recommendations

- Increasing patient awareness and understanding of PRO measures is important to improve participation and subsequently the value payers place on these outcomes.
- Increased uniformity and robustness in data collection will help to improve the value of these PRO measures and ensure data reliability. This was supported by both German payers.
- One Canadian payer thought HTAs need to have a standardized interpretation of the data from PRO measures to make it more impactful in decisions.

	Overall	US	Canada	Germany
Priority of Patient Voice	The level of importance on patient voice differed across countries.	Due to subjectivity, PROs do not have much influence in the decision-making process when compared with other parameters such as clinical outcomes (safety, efficacy).	The patient voice can impact decisions in specific situations; however, inputs are reviewed systematically and are prioritized where HRQoL is the third most important parameter. Clinical measures such as overall survival and progression-free survival (PFS) are considered more important.	PRO measures are an important criterion in the German assessment process, and there is a preference for PRO measures over clinician- or caregiver-reported data
		"We do not argue that these parameters are not validated. We are hung up at the subjectivity of variation responses."	"And it was the quality-of-life data and also the testimony of patients for what it was like to live with a spleen that's sticking out of your body and enlarged and what that means for a person's day-to-day function. It was terribly important, and it probably flipped the file."	"The patient voice here in Germany is quite important. They participate in every decision-making process and any discussion; they can put topics on the agenda."
Patient Reported Outcomes Influence on Decision-Making	Patient journey concepts can be considered for an additional benefit in Germany. These concepts are taken into consideration more so in Canada than in the US.	Concepts in the patient journey, such as symptomatic burden, caregiver burden, and patient preference are considered equally important in the decision process. However, both payers agreed that societal burden is not considered in the process and does not have any impact on policies at national or regional levels.	Patients' ability to function and their emotional state are taken into consideration during the decision-making process but do not influence any clinical or economic assessments. However, in Quebec, insights from caregivers are considered and incorporated into the economic assessments.	Payers from Germany indicated that assessment includes quality-of-life measures, but there is no rank order for concepts such as symptomatic burden, tolerability, and functional scores.
Changes in PRO reporting	Most payers do not expect major changes in the level of impact of patient-reported outcome data on access and reimbursement. However, payers noted that quality of life measure could be used as a differentiator in a heavily competitive environment where traditional efficacy measures become less distinguished.	Payers think that the decision-making process would remain unaffected, but the level of impact could change based on reliability or strength of the data.	With the increase in competition in the pharmaceutical industry, quality of life may become a deciding factor	Payers do not foresee any major change in the way drugs can gain positive access other than the mandatory requirement of clinical assessment data submissions.
		"We will continue to honor it, collect it, look at it, have an open ear. If the quality is great, if the trend is solid, etc., we will continue to listen. I don't think our process is going to change. What would only change is the strength of our decision making that is correlated to the strength of the data that is being delivered to us. So, the process isn't going to change, no."	No Quote Included.	"We are close to mandatory joint clinical assessments but given the current efforts on trying to get most of the German requirements into the European requirements, I don't think there will be much of a change."

Conclusion

The study explored the role of the patient voice and PRO measures in influencing access and reimbursement decisions for oncology drugs. Overall, the patient voice was recognized as an important part of drug development and decision making by all six payers. However, the impact of the patient voice on access and reimbursement decisions differed across countries. The impact of the patient voice was higher for Germany, then for Canada, and impact was lowest in the US. Further, patient advocacy groups play more of a role in influencing reimbursement in Germany, than in the US and Canada. Payers prefer randomized clinical trials for collecting PRO data and anticipate little change in how PROs are used in decision-making. However, as competition grows, quality of life data may become a more important differentiator.

In conclusion, while the integration of patient voices and PRO data into decision-making processes for oncology drug reimbursement has gained traction, substantial challenges remain in translating these insights into impactful outcomes. Bridging the gap between collecting patient perspectives and effectively using them in reimbursement decisions could enhance the relevance and quality of healthcare assessments to shape a more patient-centered approach in healthcare policy and access decisions.

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