Value of Patient-Reported Outcomes Data in Food and Drug Administration Labeling of Oncology Drugs to Payers in the United States

HPR236

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Background

- Patient-reported outcomes (PROs) are useful in assessing treatment experiences directly from the patient and are increasingly used to support labeling claims for anticancer therapies.[1]
- The United States (US) Food and Drug Administration (FDA) provides clear criteria for a core set of PROs for inclusion in clinical trials for anticancer therapies.[2]
- Previous evidence suggests that PRO data may be useful for formulary decisions.[3,4]
  - However, the value of PRO data included in oncology drug labeling by the US FDA and in dossiers for US payer decision-making is not well understood.[1,4]

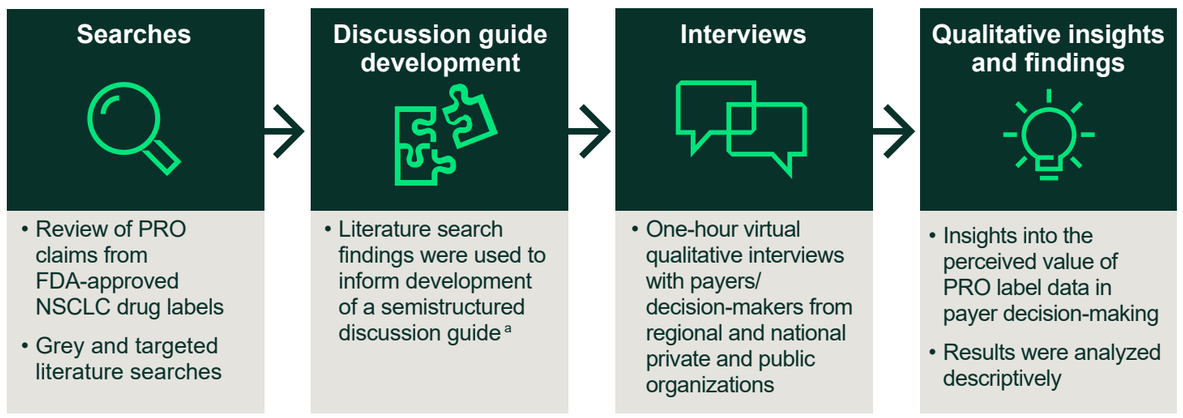
Objective

- To provide insights into how PRO-based FDA-approved product labeling statements are used by US payers when reviewing and approving new oncology drugs for formulary inclusion.

Methods

- A review of selected FDA-approved non-small cell lung cancer (NSCLC) drug labels that included PRO claims and a landscape review was conducted to yield insights into the value of PRO data to US payers when reviewing and approving new oncology drugs for formulary inclusion **(Figure 1)**.
  - The landscape review included gray and targeted literature searches to inform development of a semistructured discussion guide for qualitative interviews with US payers/decision-makers (i.e., representatives of regional and national private and public organizations).
    - Identified relevant English-language articles were first screened and subsequently reviewed by a single researcher from October to November 2024, with oncology articles that included the US payer perspective on PROs or patient experience data eligible for inclusion.
  - One-hour, virtual qualitative interviews were conducted by experienced interviewers using the semistructured guide to gain insights into the perceived value of PRO label data for oncology drugs from the perspective of US payers.
    - Participant selection targeted experienced decision-makers who are familiar with NSCLC treatments, voting members of pharmacy and therapeutics (P&T) committees or review boards who represent regional and national private and public payer organizations, and/or decision-makers involved in the review and formulary approval for NSCLC treatments.

Figure 1. Study Design



[a] Topics included roles and responsibilities, evaluation approaches to FDA-approved products with and without PRO label claims, and closing comments and recommendations.

Results

- Selected FDA-approved NSCLC drugs with PRO label data included crizotinib and selpercatinib.
- Following screening and review, 5 articles[5-9] were included in the landscape review and informed the interview guide, which facilitated discussion of (1) participants' roles and responsibilities, (2) evaluation approaches to FDA-approved products with and without PRO label claims, and (3) closing comments and recommendations.
- Six US payers (1 chief medical officer, 1 former Centers for Medicare and Medicaid Services (CMS) group head, and 4 medical directors) were interviewed individually **(Table 1)**.

Table 1. US Payer Interview Participants

Role and responsibilities	Geographic coverage area, plan type	Total (millions)	Covered lives: Commercial	Covered lives: Medicare	Covered lives: Medicaid
Chief Medical Officer (Physician, P&T committee, chair of policy committee)	National, PBM	8.5	50%	30%	20%
Former Group Head of CMS Policy and Program Alignment and former Deputy Group Head of CMS Drug and Health Plan Operations (Advisor to NCD)	National, public	NA	NA	Varies[a]	Varies[a]
Medical Director (Physician, P&T Committee)	National, MCO	100	55%	35%	NA
Global Medical Director (Clinical Pharmacist, P&T Committee)	National, MCO	10	10%	80%	10%
Senior Medical Director (Physician, P&T Committee)	Regional, IDN	21	✔	✔	NA
Medical Director (Oncologist, 1 of 4 oncology KOLs on P&T)	Regional community hospital and cancer center, IDN	NA	NA	NA	NA
Total covered lives:		**139.5 million**			

IDN = integrated delivery network; KOL = key opinion leader; MCO = Managed Care Organization; NA = Not applicable; NCD = National Coverage Determination; P&T = Pharmacy & Therapeutics; PBM = Pharmacy Benefit Manager.

Note: ✔ indicates the plan type is covered but a specific breakdown was not available.

[a] Because this is a national public plan, the numbers enrolled vary.

- Four of the 6 US payers agreed that PRO data are valued while not being a primary driver in formulary decision-making **(Figure 2)**.
  - US payers agreed PRO data from randomized controlled trials (RCTs) are considered the gold standard and that data should be published in peer-reviewed journals.
  - Payers additionally noted that FDA approval, product inclusion in National Comprehensive Care Network (NCCN) guidelines, and clinical safety and efficacy data remained the primary influencers of plan coverage and formulary status.
- Notably, US payers indicated PRO data are often part of the value proposition and connecting PRO data to cost savings may be influential. The absence of PRO data may be viewed as conspicuous or incomplete (**Figure 3**).
- US payers reported PRO data in FDA-approved labeling for US oncology drugs adds important context to the patient experience and may inform treatment selection for oncologists (**Figure 4**).

Figure 2. PROs as Part of Formulary Decision-Making for Oncology Drugs

Most interviewees agreed that PRO data are valued while not being a primary driver in formulary decision-making for oncology drugs (66.7% [n = 4/6])

"What they probably want to do is **get it into a peer-reviewed, published journal so it has validity**…"

"…**all FDA-approved drugs are covered**. […], in general the PRO data is not something that we spend a lot of time and energy focusing on. **We look at the clinical data in terms of the efficacy, for the most part.** And then safety is [secondary]."

Payer 4, Global Medical Director, National MCO

"What **resonates most** with national payers [...] that follow the **NCCN guidelines**. They're really looking at the **randomized control clinical trials that are published in peer-reviewed, esteemed clinical journals**."

Payer 3, Medical Director, National MCO

PRO data published in peer-reviewed journals are valued

Most oncology drugs are included in US plans if approved by the FDA and included in NCCN guidelines

Clinical safety and efficacy data from RCTs remain the gold standard evidence

Figure 3. PROs as Part of the Value Proposition for Oncology Drugs

"**They're part of the value story**. Usually when you say PRO to a payer, it doesn't mean much. But when you talk about **impact on function** and you talk about **toxicity, safety,** anything that has to do also with **additional resource use that impacts total cost** … but obviously meaningful to a member, meaningful to usually the provider that's trying to dialogue around options and perhaps even **convince the patient or their family around the treatment** and hopefully what life can be."

Payer 1, Chief Medical Officer, National PBM

"It's not surprising to me. Because again, they look at the compilation. Oftentimes when something is included, it's a part of and it's a compilation of it that's looked at. **When something is not included that historically or characteristically has been, the question is always why not? What are they trying to hide?"**

Payer 3, Medical Director, National MCO

PRO data have value by being often part of the value proposition

Connecting PRO data to cost savings and clinical efficacy and safety can be influential

A lack of PRO data may not negatively affect the review of the product but may be viewed as conspicuous

Figure 4. PRO Label Claims as a Driver of Oncologist Treatment Selection

"I think that **data very much influences providers**; because if they're choosing among drugs that efficacy-wise look more similar … **they're going to choose the drug with the better PRO for their patient.**"

Payer 4, Global Medical Director, National MCO

"[payers are] **allowing the physicians to make the selection.** So, they really feel that's probably who that should be directed to. Because […], **any annual update even when there have been available PRO data, has not made its way into the policy or the coverage criteria.**"

Payer 3, Medical Director, National MCO

"So, the [PRO] data will give you […] motivation to kind of fight back and most of the time **you are able to tell the payers why you insist on it** and then you will include the data when you're challenging that."

Payer 6, Oncologist, cancer center

PRO label claims may influence oncologists' treatment selections, providing important context of the patient experience

Conclusions

These findings clarified the role that PRO evidence can play in US payer decision-making for oncology drugs.

- Although US payers in this study confirmed that efficacy and safety remain the most influential, PRO data have a role in the overall evidence package and value proposition for oncology drugs.
- Oncology PRO label claims may influence oncologists' drug selections, providing important context of the patient experience.
- Linking oncology PRO data to the impact they have for payers in terms of cost savings (e.g., due to reduced healthcare resource utilization) may be influential.

References

1. Zagadailov E, et al. Am Health Drug Benefits. 2013;6(5):264-74.
2. FDA. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/core-patient-reported-outcomes-cancer-clinical-trials. Aug 2025.
3. Brogan A, et al. J Manag Care Spec Pharm. 2017;23(2):125-34.
4. Oderda G, et al. J Manag Care Spec Pharm. 2022;28(2):188-95.
5. Brixner D, et al. J Manag Care Spec Pharm. 2021;27(8):1096-105.
6. Brogan AP, et al. J Manag Care Spec Pharm. 2017;23(2):125-34.
7. Cella D, et al. Front Pharmacol. 2022;17:13:1031992.
8. Oderda G, et al. J Manag Care Spec Pharm. 2022;28(2):188-95.
9. Zagadailov E, et al. Am Health Drug Benefits. 2013;6(5):264-74.

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