Healthcare economic information (HCEI) and patient experience information (PEI) in immuno-oncology: is the research being conducted and communicated effectively to US payers?

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Acknowledgments

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BACKGROUND

Patient-focused drug development (PFDD)

- PFDD is an initiative set forth by the US FDA integrating the patient experience into the development and evaluation of emerging treatments
- Formal methodological guidance regarding data collection and dissemination for use in product development and regulatory decision-making was drafted[1]
- Despite increased attention to patient experience information by health authorities,[2] payers are challenged with how to use the information effectively, and manufacturers still struggle with generating data to meet the payers' needs

Patient experience information (PEI)

- Evidence obtained from patients regarding their experience with a disease or treatment of interest
- This could include patient-reported outcomes (PROs), patient interviews, patient journaling, patient activity tracking, and patient preferences

Healthcare economic information (HCEI)

- HCEI is any analysis that describes the economic impact of the use of a treatment
- The FDA issued formal guidance[3] in 2018 addressing communication/dissemination of HCEI to payers for approved treatments and communication with payers for products not yet approved
- Although communication of HCEI is quite clear, utilization of the information in reimbursement negotiations remains vague

OBJECTIVES/METHODS

- There are 2 primary objectives of this qualitative work:
  - To understand the impact of HCEI and PEI on innovative treatment reimbursement, with a focus on immuno-oncology (I-O) products
  - To describe information gaps with regard to HCEI and PEI from the payer perspective in order to optimize communication of results
- A strategic literature review was performed in late 2019 to identify HCEI and PEI data in support of I-O medicines
- Executive-level US payers were interviewed via double-blinded interviews (**Table 1**) to examine HCEI and PEI data in reimbursement and coverage decisions in I-O. The interviews took place in late 2019/early 2020

Table 1. Information on interviewees

Payer stakeholder	Number of stakeholders	Plan type	Covered lives (in millions)
Pharmacy Director (C-Suite/P&T Chair)	2	National (Midwest/Northeast)	40-45
Medical Director (C-Suite/P&T Chair)	2	National/Integrated (Midwest/Northeast)	5-14
Medical Director	1	Regional (West)	4

PEI RESULTS

PEI literature review

- Health authority use of PEI
  - To be convinced of treatment benefit, the FDA relies **on well-defined and reliable PRO assessments from adequate and well-controlled studies**
  - PRO labeling was granted in for **3 treatments: ibrutinib (chronic graft-vs-host disease),[4] ceritinib (non-small cell lung cancer [NSCLC]),[5] and crizotinib[6] (NSCLC) due to open-label study results in 2017**
- Examples of manufacturer use of PEI
  - Nivolumab and pembrolizumab studies examined symptom burden, physical functioning, and health-related quality of life (HRQOL) using validated PRO instruments, which were presented in national publications
  - Exploratory analyses in nivolumab studies found positive correlations suggesting that patient-reported symptoms at treatment initiation may have clinical value[7,8]

When asked whether there was additional information that could help payers utilize PEI data more effectively, the respondents indicated:

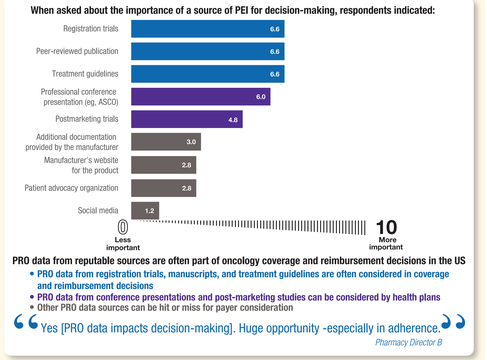
- Payers are looking for education and meaningfulness of PEI data
  - Explanation of PRO scales with more detail regarding domains and questions
  - Explanation on how to interpret change scores with greater detail regarding clinically meaningful change
  - Develop a more compelling economic story from a total cost of care perspective that includes clinical effectiveness and toxicity/HRQOL

"PROs and scales are being presented [to us]. The problem is there are so many scales but we are not familiar with these. [What would be helpful is to] 1) List all domains and questions being asked of patients 2) Provide a validation - show and define meaningful clinical importance... what does a two-point change mean?"

Medical Director E

PEI interviews

Figure 1. Relative importance of relevant PEI sources



HCEI RESULTS

HCEI literature review

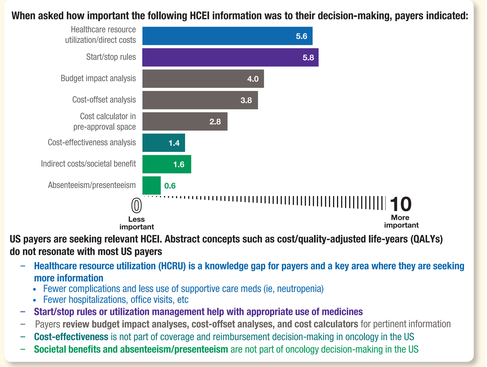
- Survival patterns and cost burden can be impactful in portraying the value story for emerging treatments
- Manufacturers have developed compelling HCEI stories by identifying cost drivers and shaping the narrative. Examples:
  - Despite higher total costs of care for second-line (2L) NSCLC, an economic study of pembrolizumab demonstrated lower weekly costs for the progression-free health state[9]
  - A study of nivolumab in patients with 2L NSCLC showed that costs associated with management of adverse events were more expensive in the control arm than in the treatment arm[10]

When asked what type of additional information could help payers utilize HCEI data more effectively, respondents indicated:

- Oncology HCEI will need to be communicated more effectively
  - Although HCEI data were identified in the targeted literature search, there is a need for HCEI data in oncology other than budget impact models
  - Economic stories based on HCEI from manufacturers are emerging, but payers have not seen much compelling economic evidence presented on I-O therapies
  - Payers would like to see more long-term follow-up HCEI with key I-O therapies
  - HCEI data are disparate and do not provide an adequate, comprehensive picture of total cost of care
  - Payers do not believe HCRU offsets will cover the cost of novel oncology therapies, but cost offsets could be a differentiator as the competitive environment increases

HCEI interviews

Figure 2. Relative importance of different types of HCEI



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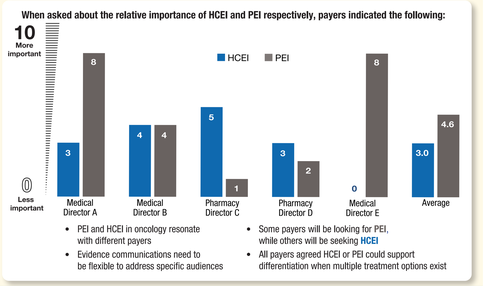
Payers' perceptions and needs when interpreting and using HCEI and PEI

Figure 3. Relative importance of HCEI vs PEI



CONCLUSIONS

- **Communication of HCEI and PEI in clear, concise ways and in accordance with FDA guidance is critical for US payers to consider in their decision-making**
- **Although there are limited therapies with PRO data in the label, the FDA is more pragmatic and flexible in assessing PROs and manufacturers can supplement PFS data when OS is immature**
- **Despite PEI becoming more relevant, it is not perceived as the most important decision factor for payers. Efficacy is the key driver, followed by safety. Patient symptoms and functioning will only be considered thereafter**
- **Payers would like to have more diverse economic data available for decision making**
- **All payers agreed that HCEI or PEI could support differentiation where efficacy and safety are comparable among treatment options and can help justify continued treatment after progression**
- **Evidence communications need to be flexible to address specific audiences. Interactive, visually appealing summaries of peer-reviewed publications that tell compelling value stories from a total cost of care perspective have the most impact on payer decision-making**

DISCLOSURES

APB reports employment at AESARA, Chapel Hill, NC and research grants from EMD Serono. **CKP** reports employment at AESARA, Chapel Hill, NC. **NS** reports employment at Merck KGaA, Darmstadt, Germany. **SB** reports employment at EMD Serono Research & Development Institute, Inc., Billerica, MA, and ownership interest at Bristol Myers Squibb.

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