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OPINIONS OF DECISION-MAKERS ON THE CLINICAL DEVELOPMENT AND ASSESSMENT OF ANTINEOPLASTIC AGENTS

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INTRODUCTION

Regulatory early access routes in the European Union (EU) have increased flexibility in the authorisation of promising treatments.[1,2] Health Technology Assessment (HTA) bodies tend to request mature clinical data sets for economic and relative efficacy assessments.[2,3,4] Industry stakeholders have indicated that oncology products are associated with divergences between regulatory and HTA clinical assessments[4] potentially hindering access to novel medicines.

AIMS

To explore and compare regulatory and HTA expert opinions on the following aspects related to antineoplastic agents:

- The quality of clinical evidence generated,
- The alignment of clinical evidence needs between regulatory and HTA decision-makers
- The impact of improving the regulatory-HTA interface.

METHOD

Tool Development

- Online survey
- Survey items:

i. 5-point agreement scales:
1 (Strongly disagree) → 5 (Strongly agree)

ii. 5-point quality scales:
1 (Poor quality) → 5 (Excellent quality)

iii. Rank-type questions

Psychometric Evaluation of Tool

- Validation: Content Validity Index (CVI) method with a panel consisting of clinical (n=4), regulatory (n=1), HTA (n=2) and informatics (n=1) specialists
- Intra-subject reliability: test-retest approach (2-week interval)

Recruitment of Study Participants

- Non-probability, purposive sampling
- Oncology experts recruited from EU HTA bodies and the European Medicines Agency (EMA)

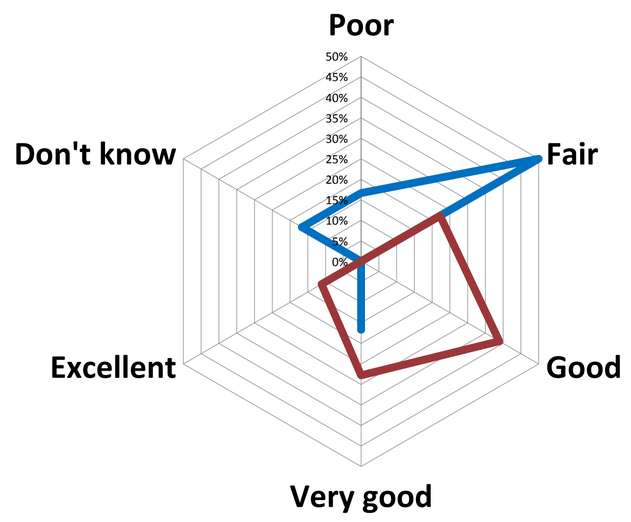
Statistical Analysis

- Descriptive and inferential statistics
- Weighted mean ratings for ordinal scales analysed using the Mann-Whitney U Test

RESULTS

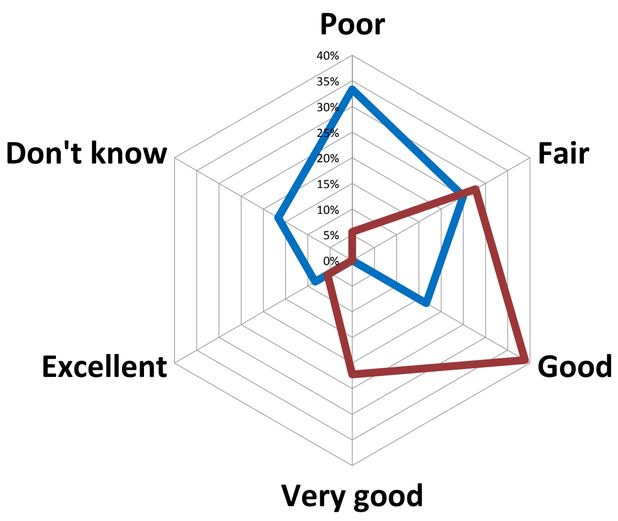
- Twelve HTA experts from 9 different EU countries with optimal geographical distribution and 18 regulatory representatives from the EMA completed the survey questions.
- HTA respondents consider that the clinical evidence requested by their agency for antineoplastic therapies is similar to that of other HTA bodies and divergent to the requirements of the EMA (p-value < 0.01).
- Decision-makers expressed significantly conflicting views on the quality of evidence generated for antineoplastic agents in the pre- and post-authorisation phases. The radar graphs in Figures 1A and 1B demonstrate that regulatory opinions are skewed towards higher quality data.
- From a list of 6 stakeholders, both groups of decision-makers ranked patients as the top stakeholder to benefit from enhanced regulatory-HTA synergy throughout the medicinal product life cycle.

(A)



Decision-maker group	Weighted mean rating
HTA	3.3
Regulatory	2.2
p-value	0.01

(B)



Decision-maker group	Weighted mean rating
HTA	2.9
Regulatory	2.1
p-value	0.04

Figure 1. Decision-maker opinions on the quality of evidence generated for antineoplastic agents in the: (A) pre-authorisation phase, (B) post-authorisation phase

CONCLUSION

Expert opinions indicate that clinical evidence needs for antineoplastic agents are not optimally aligned between regulatory and HTA bodies and that the quality of evidence generated is perceived differently. Decision-makers recognise patients as the main stakeholders to benefit from increased regulatory-HTA interactions. Findings from this study are intended to stimulate calls for effective harmonisation between the two facets, potentially driving faster patient access to innovative cancer treatments.

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