

Stakeholder survey of Broad elements of Value in Health Technology Assessment in Australia.

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INTRODUCTION AND AIM

To investigate Australian stakeholder opinions about including broader elements of value (Figure 1) in economic evaluations in HTA in Australia for treatments of rare diseases.

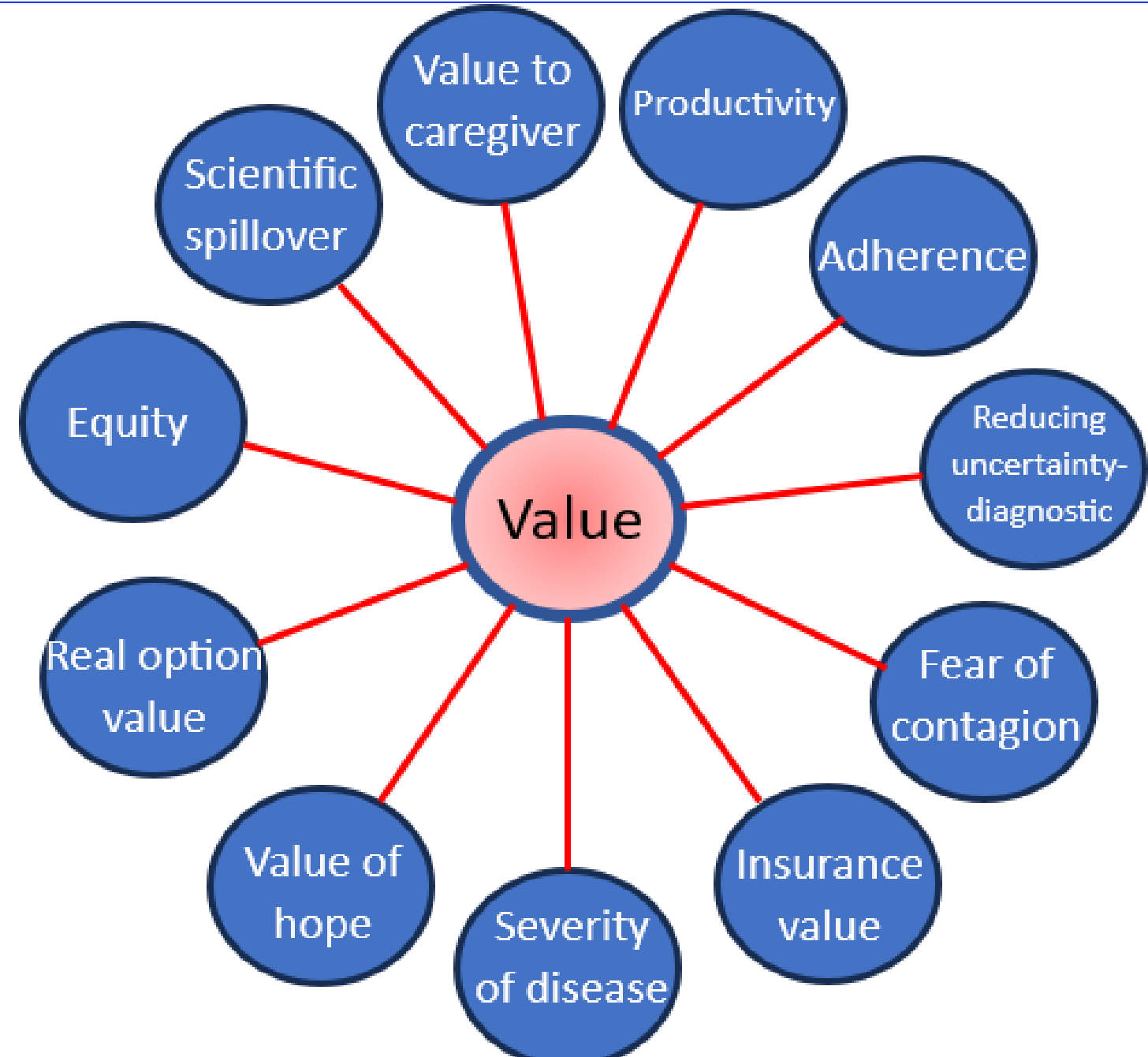


Figure 1.Elements of value adapted from Lakdawalla et al. 2018.

METHODS

Stakeholders involved in HTA in Australia, representing academia (n=11), specialist consultants (n=10) and the pharmaceutical industry (n=23) participated. The online survey was conducted between October 2023 to May 2024. Data analysis was based on descriptive statistics and chi-square comparisons.

RESULTS

Table 1. Stakeholder view and comparison on HTA method adequacy and transparency

| Q:Do you think the current HTA methods applied in Australia are adequate to appropriately assess the cost effectiveness of all medicines? n/N (%) Agree | |
|--|-------------|
| Total cohort | 5/43 (12%) |
| Academia | 3/11 (27%) |
| Private sector | 2/32 (6%) |
| Q:Do you think the current HTA methods applied in Australia are adequate to appropriately assess the cost effectiveness of medicines for rare diseases? n/N (%) Agree | |
| Total cohort | 8/44 (18%) |
| Academia | 2/11 (18%) |
| Private sector | 6/33 (18%) |
| Q:Do you agree that the current public information regarding reimbursement decisions in Australia provides sufficient information about which sources of value are considered and how they contributed to decision-making, n/N (%) Agree | |
| Total cohort | 11/41 (27%) |
| Academia | 5/11(45%) |
| Private sector | 6/30 (20%) |
| Q:Do you agree that an explicit checklist of sources of value beyond the patient QALY and whether they were considered by decision maker would be more informative than what is currently published in Australia? n/N (%) Agree | |
| Total cohort | 28/40 (70%) |
| Academia | 5/10 (50%) |
| Private sector | 23/30 (77%) |

DISCUSSION

There was considerable agreement in responses between academia and private sector with no statistically significant differences (p>0.05) between responses in relation to sector for any of the questions based on a Chi-square test.

Most (>80%, Table 1) respondents agree that current HTA methods applied in Australia are inadequate to assess the cost effectiveness of medicines generally and medicines for rare disease, and that current public information on sources of value considered in decision-making is insufficient. Greater transparency is required on the factors affecting HTA decision making as it enables stakeholders to collect relevant data to inform decision making .

Most (70%, Table 1) respondents agree the inclusion of a checklist of the health and non-health effects of therapy considered as part of HTA would help ensure that all consequences are considered explicitly and transparently in decision making.

Less than 20% of Australian stakeholders agree that fear of contagion and insurance value should be considered in HTA of all medicines or medicines for rare disease. Of the six broad value elements that more than half (50%) of Australian stakeholders think should be considered in HTA of medicines generally and for medicines for rare disease in Australia (Table 2); only two (disease severity and equity) overlap with the “less -readily quantifiable” factors that are quoted to influence PBAC decision making in Australia. The PBAC guidelines are not prescriptive, noting alternative approaches are permitted. As such alternative value elements can be included in submission but requires decision makers to be clear about their opinion on whether the value element was justified and well-supported. Such clarity is important for sponsors given the time and investment required to develop adequate scientific evidence that recognizes the wider benefits of therapy.

The largest difference, albeit not statistically significant, between stakeholder views was for the value of hope, a larger proportion of private sector respondents agreed that it should be considered compared with those in academia (43% and 20%, respectively for all medicines, 27% versus 10% for medicines for rare disease).

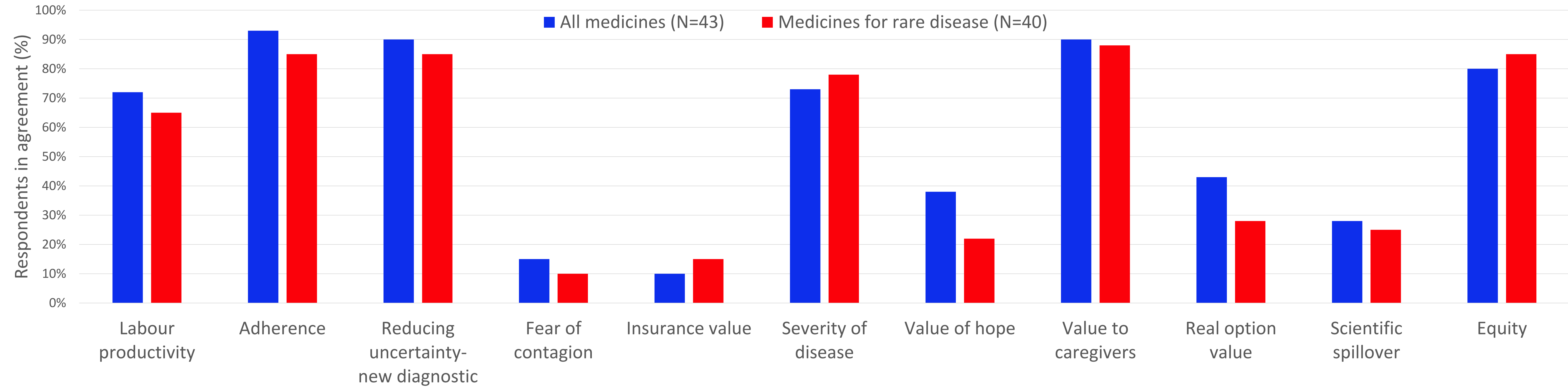
CONCLUSIONS

Australian stakeholder views were similar and did not diverge between medicines generally and for rare disease. Stakeholders agree that methods are not adequate for HTA of medicines for rare and non rare disease in Australia and public statements lack transparency on which source of value contributed to reimbursement decision. Stakeholders favoured broader value elements in HTA than referred to in the PBAC guidelines. Further advice in reimbursement guidelines and transparency in publication of decisions regarding the values affecting decision-making is needed.

References

Lakdawalla DN, et al. Defining Elements of Value in Health Care-A Health Economics Approach: An ISPOR Special Task Force Report [3]. Value Health. 2018;21(2):131-9.
Qiu T, et al. Challenges in the market access of regenerative medicines, and implications for manufacturers and decision-makers: a systematic review. Regen Med. 2022 Mar;17(3):119-139.

Table 2: Which sources of broader value should be included in HTA in Australia



Acknowledgments

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