THE IMPACT OF CLINICAL EVIDENCE ON THE INCLUSION OF NON-SMALL CELL LUNG CANCER DRUGS IN BRAZIL'S SUPPLEMENTARY HEALTHCARE SYSTEM

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OBJECTIVES

This study aims to evaluate the impact of clinical evidence in the evaluation of non-small cell lung cancer (NSCLC) drugs submitted for inclusion in the listings of Brazil's National Supplementary Health Agency (ANS) since October 2021.

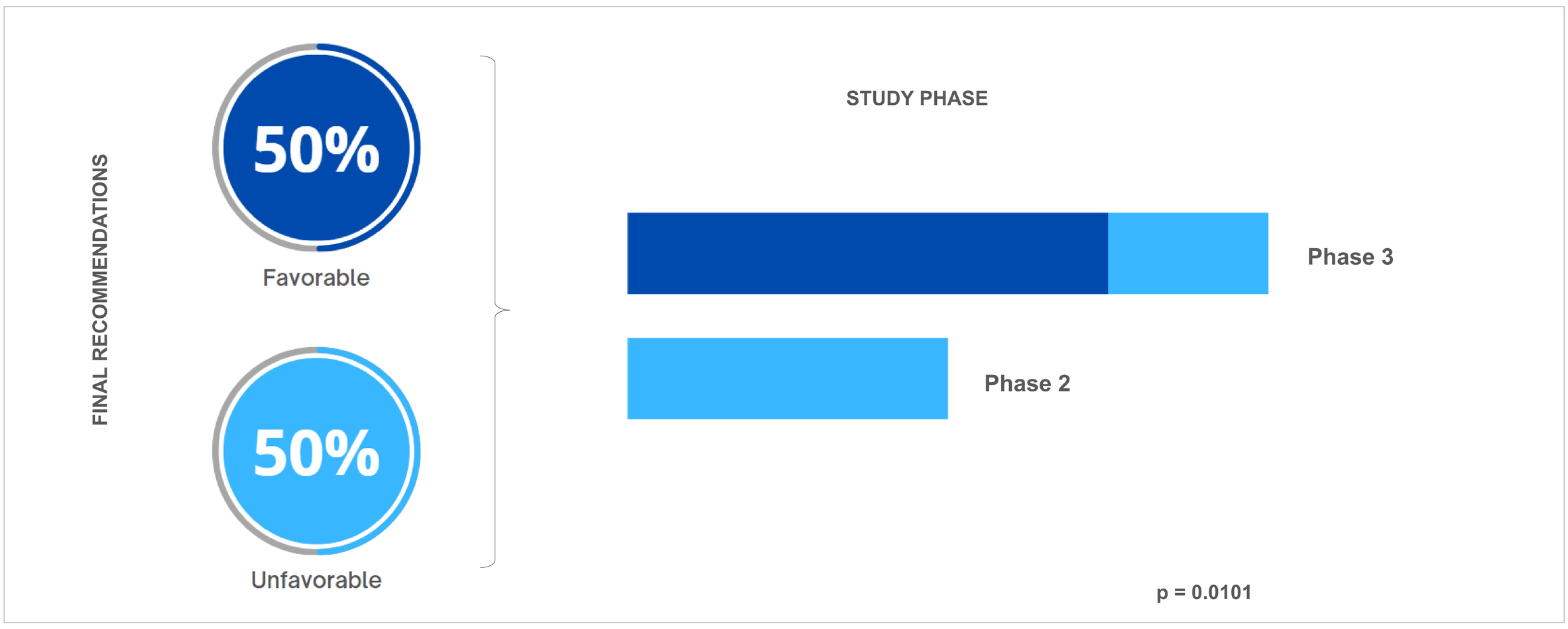
METHODS

On June 25, 2025, a review of all NSCLC drug submissions for inclusion in the ANS list since October 2021 was carried out. Technologies were grouped by presence or absence of Phase 2 and Phase 3 trials, and two 2x2 contingency tables were constructed: one comparing the presence of Phase 3 evidence with the recommendation outcome, and another for Phase 2 evidence. To assess the association between evidence type and recommendation outcome, Fisher's exact test was applied with Monte Carlo simulation (10,000 replicates) to account for the small sample size and zero cells in the contingency tables. In addition, Haldane-Anscombe correction was used to compute adjusted odds ratios (ORs) and 95% confidence intervals (CI), by adding 0.5 to all cells to allow estimation in cases of complete separation. All analyses were conducted using R (version 4.5.1).

RESULTS

Six submissions were identified. Three received positive final recommendations: brigatinib, lorlatinib as a first-line treatment (1L), and osimertinib. The negative recommendations included lorlatinib as a second-line treatment (2L), tepotinib, and selpercatinib. All unfavorable recommendations were supported primarily by phase 2 studies, although selpercatinib also had phase 3 data. In contrast, all favorable recommendations were based on phase 3 trials, with network meta-analyses (NMAs) additionally presented for brigatinib and lorlatinib (1L). Fisher's exact test indicated a statistically significant association between study phase and ANS decision (p = 0.0101). Phase 2 studies were more likely to be rejected compared to phase 3 studies; however, this difference was not statistically significant (odds ratio = 0.02; 95% CI: 0–1.348) (Figure 1).

Figure 1. Association Between Trial Phase and ANS Evaluation Outcomes.



Source: Developed by the authors.

CONCLUSION

These findings highlight the importance of clinical evidence quality in the assessment of NSCLC medications by the ANS in Brazil. Submissions based on Phase II trials with high risk of bias and low certainty of evidence may face challenges in gaining approval.