

# Comprehensive Clinical Evaluation of Amphotericin B in the Treatment of Invasive Fungal Disease in China

HTA86

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## BACKGROUND

- Invasive fungal disease (IFD) causes over 300 million severe cases and 1.5 million deaths annually worldwide, being a leading cause of mortality in patients with hematological malignancies.
- Global antifungal resistance is on the rise. Furthermore, conventional amphotericin B deoxycholate (AmBd) is associated with significant nephrotoxicity. Lipid formulations improve safety but lack comprehensive HTA evidence for rational selection—especially in middle-income countries like China.

## OBJECTIVE

- To develop a clinical comprehensive evaluation index system for the treatment of invasive fungal disease and to conduct empirical research to provide methodological references for rational clinical use in hospitals.

## METHOD

- Based on literature review and relevant guidelines<sup>[1-3]</sup>, the Delphi method was employed to construct a clinical comprehensive evaluation index system and evaluation criteria for the treatment of invasive fungal infections.
- AmBd, liposomal amphotericin B (L-AmB), and amphotericin B colloidal dispersion (ABCD) were included as examples in the empirical research.

## RESULTS

### Clinical Comprehensive Evaluation Index System

6 primary indicators and 13 secondary indicators were finalized along with their corresponding weights and quantitative scoring.

Table 1. Comprehensive Clinical Evaluation Index System for IFD Medications

| First-Level Index | Second-Level Index                            | Weight |
|-------------------|---|--------|
| Safety            | A1- Incidence and severity of adverse events  | 12%    |
|                   | A2- Drug contraindications                    | 11%    |
| Effectiveness     | B1- Authoritative guideline recommendations   | 22%    |
|                   | B2- Therapeutic effect observation indicators | 18%    |
| Economy           | C1- Drug price                                | 7%     |
|                   | C2- Cost-utility analysis                     | 7%     |
| Innovation        | D1- Mechanism innovation                      | 3%     |
|                   | D2- Application innovation                    | 3%     |
| Appropriateness   | D3- domestic original research and innovation | 2%     |
|                   | E1- Technical appropriateness of the drug     | 3%     |
| Accessibility     | E2- Appropriateness of drug use               | 4%     |
|                   | F1- Drug accessibility                        | 4%     |
| Total             | F2- Affordability                             | 4%     |
|                   |   | 100%   |

### Comprehensive Scores of Three AmB Formulations

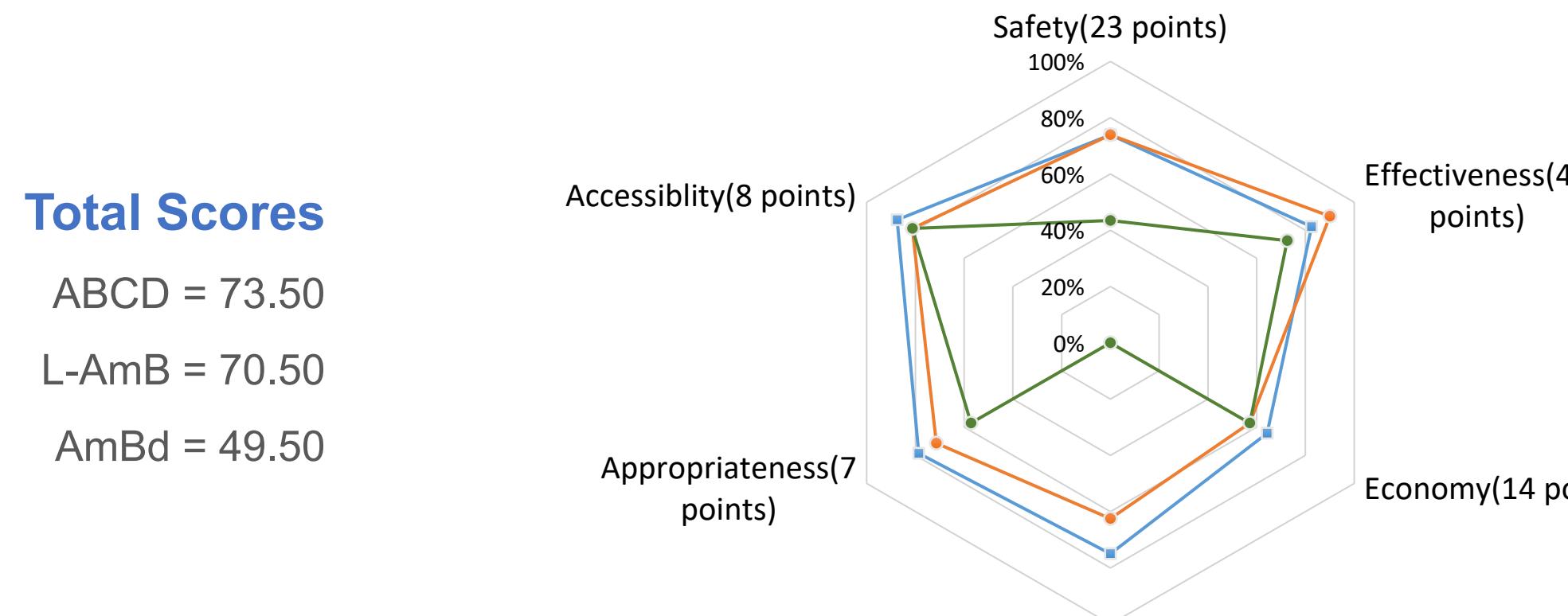


Figure 1. Radar Map of Clinical Comprehensive Evaluation Scores

### Dimension-Specific Results

#### Safety

- The three amphotericin B formulations show no significant differences in fever, chills, discontinuation rate, mortality, overall adverse events, liver injury, or infusion reactions. However, ABCD and L-AmB have lower nephrotoxicity than AmBd; ABCD is safer for patients with impaired hepatic or renal function, while AmBd carries stricter usage restrictions in cases of severe organ dysfunction<sup>23</sup>.

#### Effectiveness

- L-AmB demonstrated superior efficacy and received higher recommendation grades in both domestic and international guidelines.

#### Economy

- Compared with AmBd, the ICERs for both ABCD and L-AmB were below the willingness-to-pay (WTP) threshold of one times the GDP per capita in China.

#### Accessibility

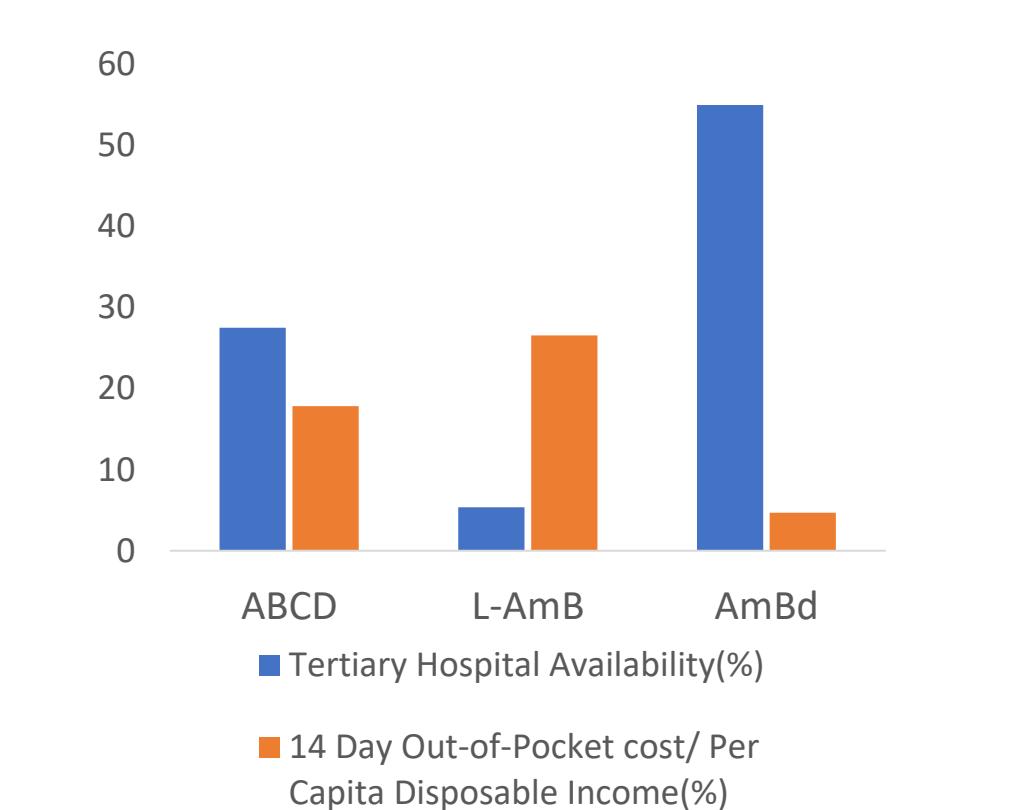


Figure 2. Accessibility and Affordability of Three AmB Formulations

#### Innovation

- As a Category 4 Priority New Drug in China, ABCD addresses a clinical gap by allowing dose escalation from 1 mg/kg to 6 mg/kg due to its lower nephrotoxicity, thereby shortening treatment, improving efficacy, and enhancing compliance, particularly for patients intolerant or unresponsive to AmBd.

#### Appropriateness

- ABCD, stable at room temperature with a 2-hour infusion, offered practical advantages over L-AmB and the more cumbersome AmBd, which requires refrigeration and a prolonged (>6 hours), light-protected infusion

## CONCLUSIONS

The proposed clinical evaluation index system offers a structured and evidence-based approach for guiding antifungal drug selection. The comprehensive evaluation, which integrates safety, efficacy, economy, and other dimensions, revealed a trade-off: ABCD provided the best overall value due to its favorable economic profile and safety advantages, while L-AmB offered the highest clinical efficacy. These findings can inform value-based decision-making in clinical practice.

## REFERENCES

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