



Study on the Accessibility of Orphan Drugs in China's Urban Customized Commercial Medical Insurance

Xinting Liu 1, Zhihao Zhao 1, Yangyang Fan 1, Ming Hu 1#

1 West China School of Pharmacy, Sichuan University, Chengdu, China.



Background and Objective

- As part of China's multi-level healthcare security system, Urban Customized Commercial Medical Insurance (UCCMI) provides supplementary coverage for orphan drugs.
- This study aims to evaluate the characteristics and accessibility of orphan drugs involved in UCCMIs.

Methods

- Data were obtained from the PHARMCUBE database.
- Orphan drugs were defined as medicines for treating rare diseases identified in China's National Rare Disease Catalog, and those covered by UCCMI during 2020-2024 were included in the analysis.
- Descriptive statistical analysis was conducted to summarize key characteristics. Specialty drug coverages set the deductible, reimbursement rate, and cap.
- Most common orphan drugs (≥ 5 inclusions in 2024 UCCMIs) were included in analyses of accessibility and affordability.
- Affordability ratios were defined as the out-of-pocket cost per patient divided by the 2024 urban household non-food consumption expenditure.
- Availability ratios were defined as the proportion of orphan drugs included in regional UCCMIs.

Results

- 124 orphan drugs were analyzed, corresponding to 115 UCCMI products and 208 Specialty drug coverages.
- 51.61% (64 drugs) were listed in the National Reimbursement Drug List (NRDL).
- Hemophilia was the most frequent indication (10 drugs).
- The most typical Specialty drug coverage (127, 61.06%) directly set the deductible and cap (¥0 and ¥1,000,000), with reimbursement rates differentiated for pre-existing (30.00%) and non-pre-existing (80.00%).

Results (continued)

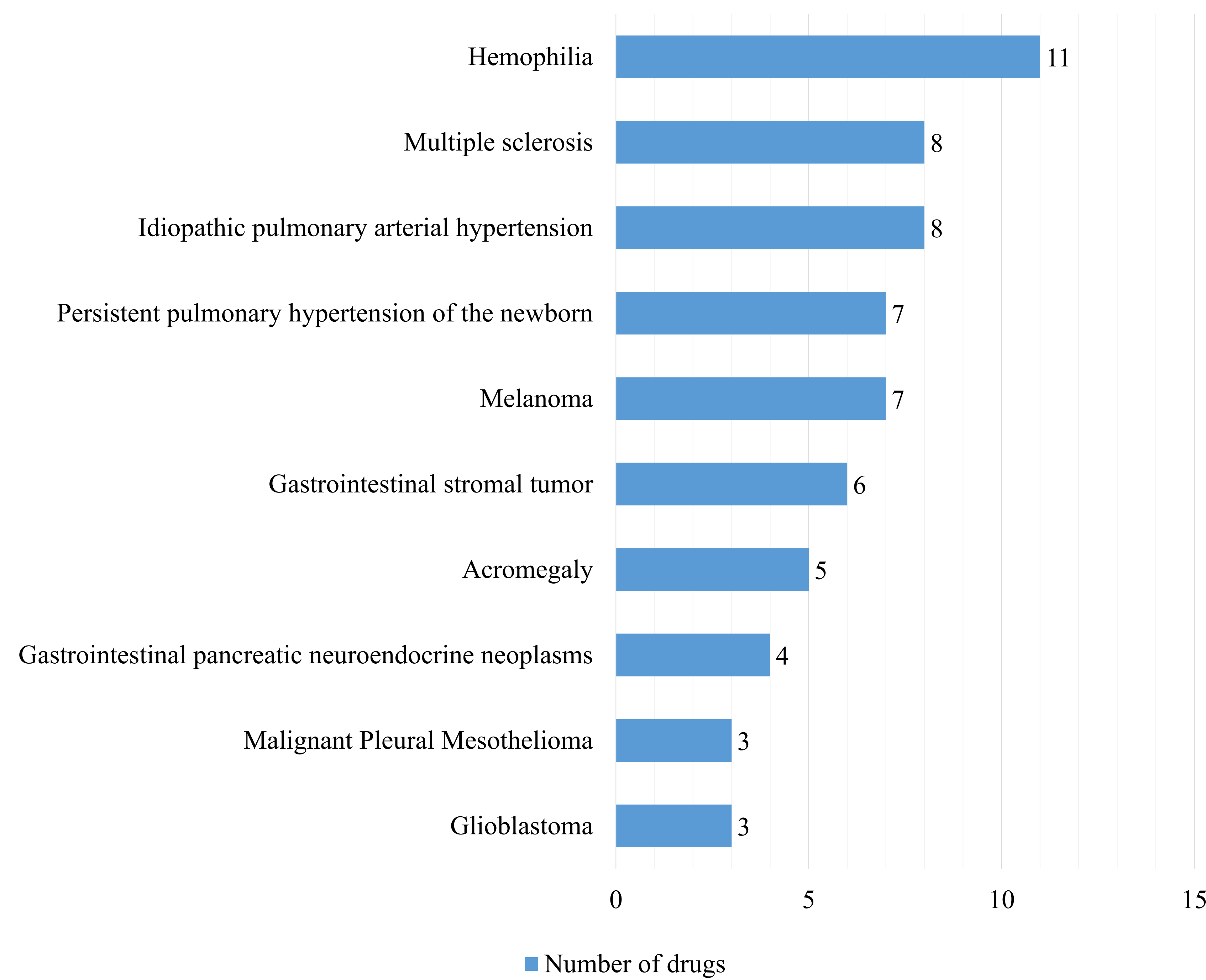


Fig 1 Top 10 Orphan Drug Indications by Number of Drugs

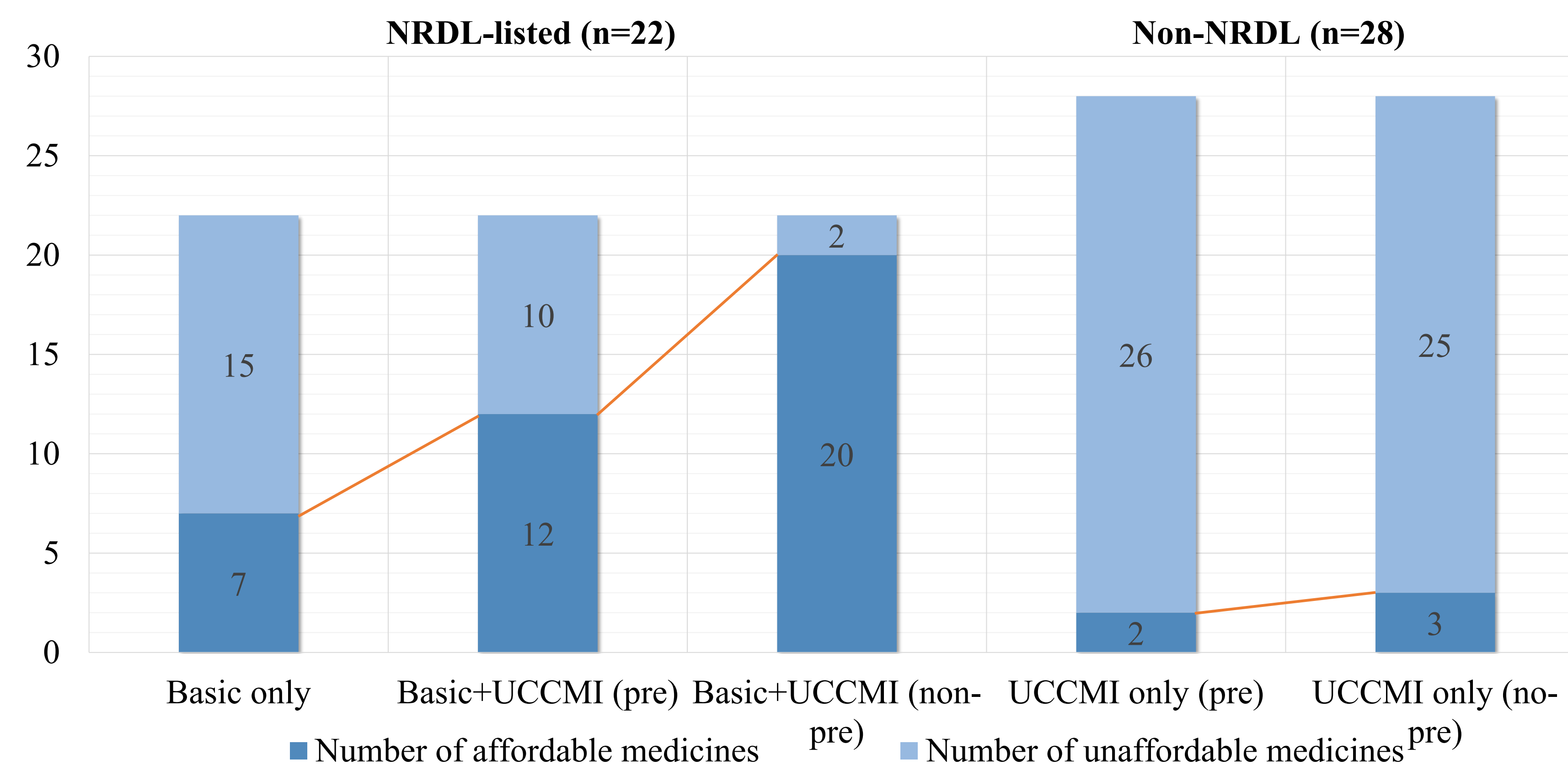


Fig 2 Number of affordable orphan drugs under different reimbursement scenarios

Results (continued)

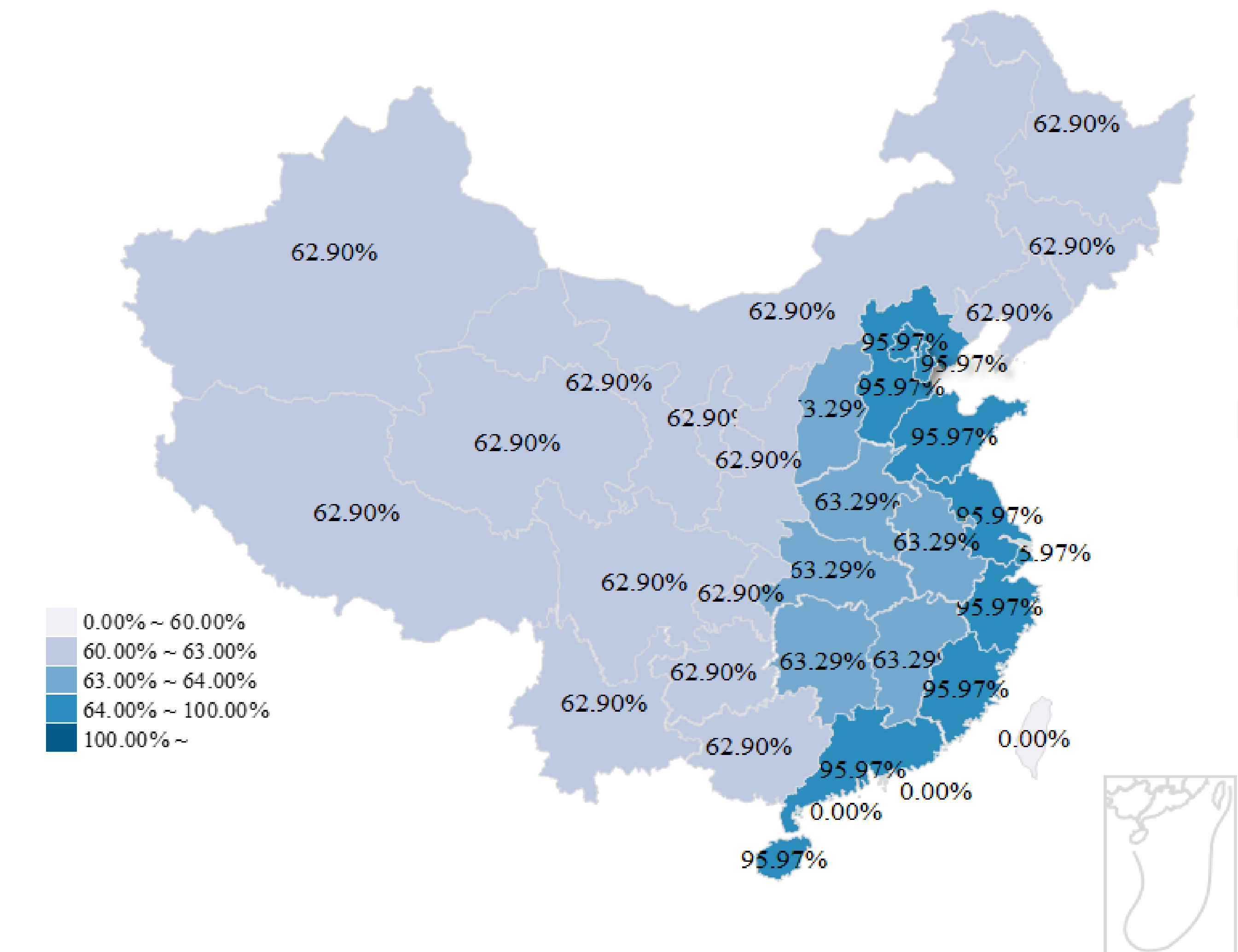


Fig 3 UCCMI coverage for orphan drugs across China's four regions

- Among 22 NRDL-listed orphan drugs most frequently covered by UCCMI, the affordable drugs rose from 7 to 20 (non-pre-existing) and to 12 (pre-existing) after being reimbursed by both basic medical insurance and UCCMI.
- Among the remaining 28 drugs, which were not listed in NRDL, only 3 (non-pre-existing) and 2 (pre-existing) were affordable when reimbursed solely through UCCMI.
- UCCMI coverage ranged from 95.97% of orphan drugs in the eastern region to 62.90% in the northeastern region.

Conclusion

- UCCMI has enhanced the accessibility of orphan drugs in China through diversified Specialty drug coverage; however, coverage remains insufficient for non-reimbursed drugs, and availability is uneven across regions.

Presented at ISPOR 2026, May 17-20, 2026, Philadelphia, PA, US