HPR14

Accessibility of Full versus Partial Orphan Drugs in China: An Analysis Based on the 2017-2024 National Drug Price Negotiation

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Background and Objective

- Rare diseases, despite their low individual prevalence, collectively affect a substantial proportion of the population and impose a considerable burden on patients, families, and healthcare systems.
- This study aimed to compare the characteristics and accessibility between Full Orphan Drugs (only orphan indications) and Partial Orphan Drugs (orphan and non-orphan indications) incorporated through China's National Drug Price Negotiation from 2017 to 2024.

Methods

- **Data sources:** A retrospective database was constructed using public data from government portals, Pharmcube and YaoZH platform.
- **Study sample:** Drugs with indications covering diseases in China's First and Second National Rare Disease Catalog were included.
- **Data Analysis:**
  - Accessibility was assessed in accordance with the WHO/HAI methodology for measuring availability and affordability.
  - Availability was calculated as the proportion of healthcare institutions or retail pharmacies stocking the relevant rare disease medications relative to their national representation.
  - Affordability was expressed as the ratio of daily out-of-pocket (OOP) expense to China's 2025 minimum daily wage.
  - Descriptive statistics were used to summarize key characteristics, and Mann-Whitney U tests were employed to compare accessibility between the two groups.

Results

- 71 orphan drugs were identified, including 47 Full Orphan Drugs and 24 Partial Orphan Drugs.
- The identified drugs covered 44 rare diseases, with multiple sclerosis and idiopathic pulmonary arterial hypertension (7 each) being the most common for Full Orphan Drugs, while melanoma (4) for Partial Orphan Drugs.

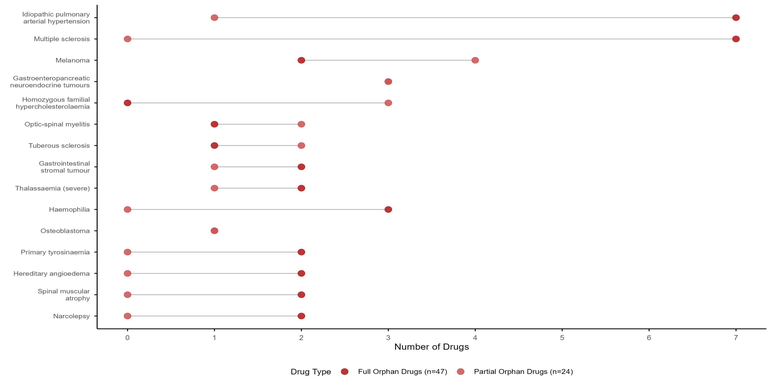

Figure 1 Drug coverage counts for the top 15 rare diseases

- Full Orphan Drugs consistently exhibited higher affordability ratios both before (3.863 vs. 2.77) and after reimbursement (1.159 vs. 0.831), but the difference was not statistically significant (p=0.3159).

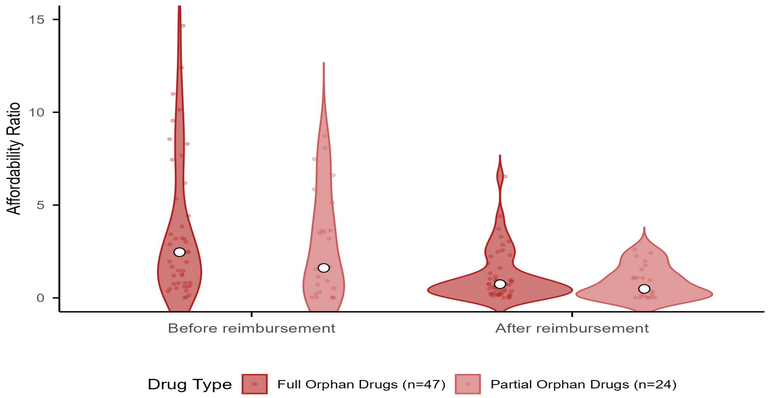

Figure 2 Distribution of affordability before and after reimbursement

- The hospital availability in Partial Orphan Drugs was significantly higher (0.065 vs. 0.026, p<0.05), whereas retail pharmacy availability did not differ significantly.

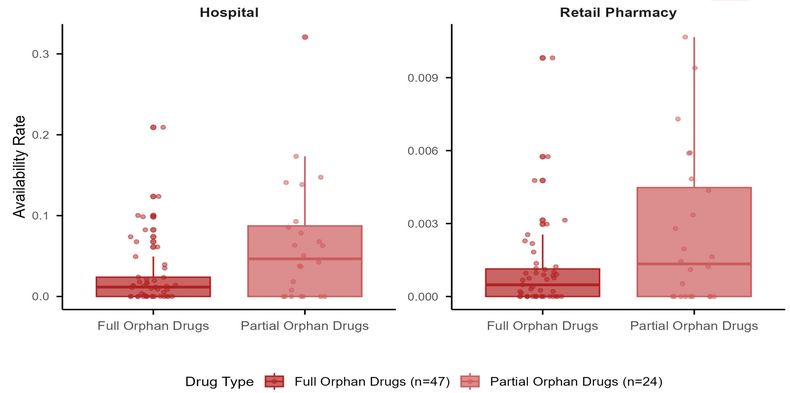

Figure 3 Hospital and retail pharmacy availability of full vs partial orphan drugs

- Partial Orphan Drugs received higher rates of expedited approval policy support (83.3% vs. 76.6%).

Table 1 Support rates for expedited approval policy and group comparisons

Expedited approval policy	Full Orphan Drugs(n=47)	Partial Orphan Drugs(n=24)	*p* value
Breakthrough therapy designation	3(6.4%)	4(16.7%)	0.216
Priority review and approval	36(76.6%)	19(79.2%)	1.000
Conditional approval	5(10.6%)	10(41.7%)	0.005
At least one expedited approval	36(76.6%)	20(83.3%)	0.759

Conclusions

- Partial Orphan Drugs demonstrated better hospital availability, likely due to their broader indications.
- However, Full Orphan Drugs remained less affordable and faced greater access barriers in healthcare settings.
- Policies should prioritize enhancing institutional supply and affordability support for Full Orphan Drugs.

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