



Characteristics and Accessibility of Orphan Drugs Through 2017-2024 National Drug Price Negotiation in China

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Session 1



Background and Objective

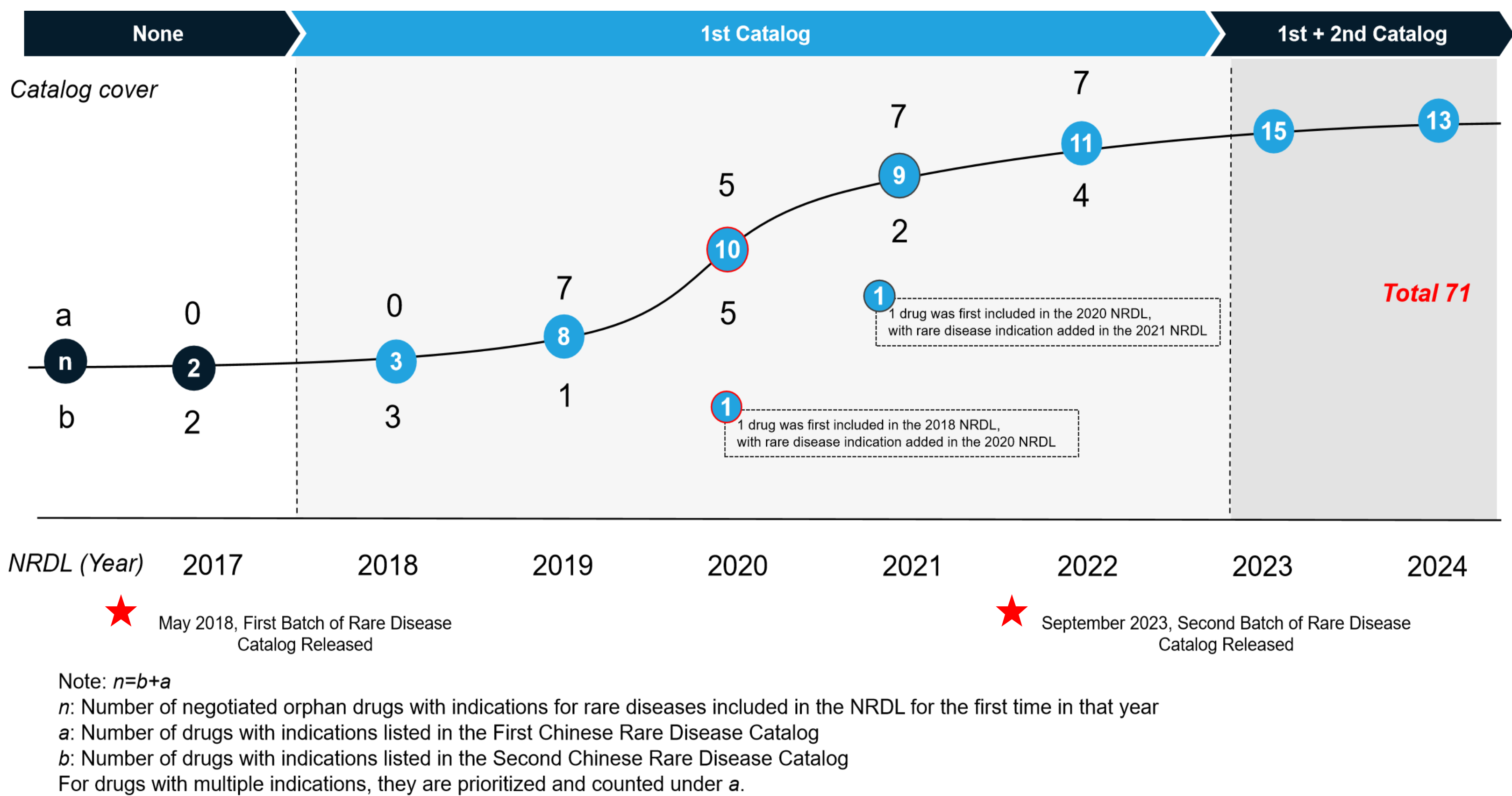
- Rare diseases (orphan diseases) are characterized by low prevalence but high disease burden, and have become a major global public health challenge.
- China has improved orphan drug access through the establishment of two batches of rare disease catalogs, the dynamic adjustment of the National Reimbursement Drug List (NRDL). Also, China has also actively explored multi-level security systems.
- Orphan drugs still face multiple challenges, including sustainability pressures on the medical insurance fund and insufficient coordination among multi-level security systems.
- To better address these challenges, it is necessary to systematically review and evaluate the basic characteristics and accessibility of orphan drugs currently included in the NRDL. based real-world data of 2017-2024 National Drug Price Negotiation (NDPN), this study aims to:
 - (1) analyze the access status and characteristics of orphan drugs included in basic medical insurance, providing references for the inclusion of orphan drugs in the commercial insurance innovative drug list;
 - (2) evaluate the accessibility of orphan drugs in the NRDL to generate evidence-based support for policy-making.

Methods

- Data Source:** A retrospective database was constructed by integrating data from government portals, Pharmacube and YaoZH Databases.
- Study Sample:** The rare diseases were defined as those listed in the first and second batches of China's National Rare Disease Catalog. Orphan Drugs through NDPN included in this study refer to those negotiated for inclusion during this period, whose reimbursement scope under the NRDL covers the prevention, diagnosis, and treatment of corresponding rare diseases.
- Statistical Analysis:**
 - Descriptive statistical analysis** was applied to key characteristics.
 - Affordability ratio:** Referring to the WHO/HAI affordability calculation methodology, our study firstly figured out the drug cost per DDD (DDDC) based on negotiated prices and defined daily doses (DDD). DDD values came from the WHO ATC/DDD Index and package inserts, and price data from the YaoZH Database. Then we calculated the out-of-pocket (OOP) of each drug and treatment category for patients after reimbursement based on the median reimbursement rates for urban employees and rural and urban residents in China. Affordability ratios were measured using China's 2024 minimum daily wage as the benchmark, with a ratio ≤ 1 being seen as affordable.
 - Availability ratio:** The availability ratio for a specific orphan drug is defined as the number of healthcare institutions/retail pharmacies stocking the drug divided by the total number of institutions/pharmacies covered by the listed drugs.

Results-1

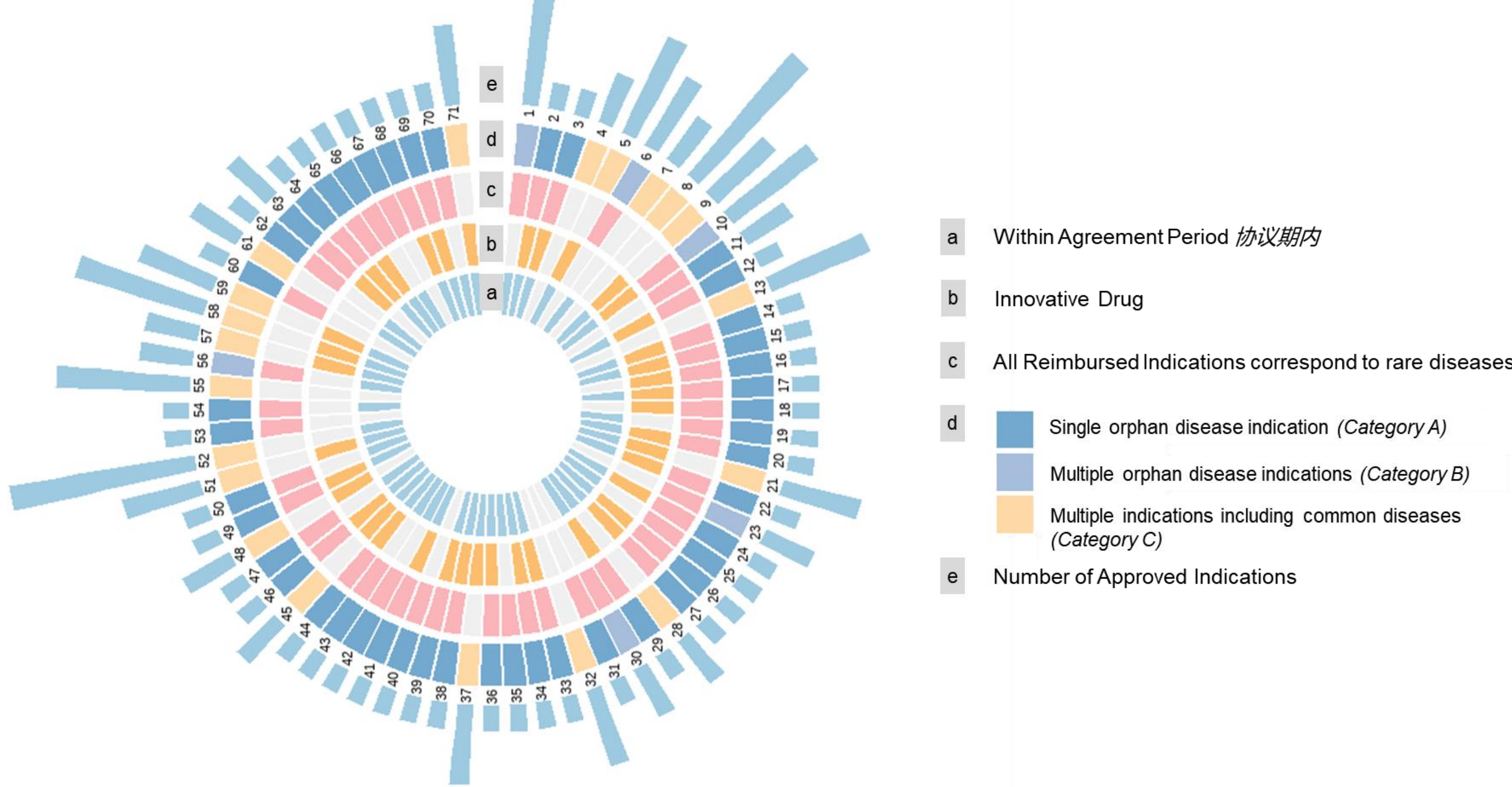
Figure 1 Timeline of NRDL Inclusion Orphan Drugs in China



- 71 listed orphan drugs were analyzed, covering 48 rare diseases indications and 20 treatment categories. The annual inclusion of orphan drugs increased from an average of 6 (2017-2021) to 13 (2022-2024) annually.

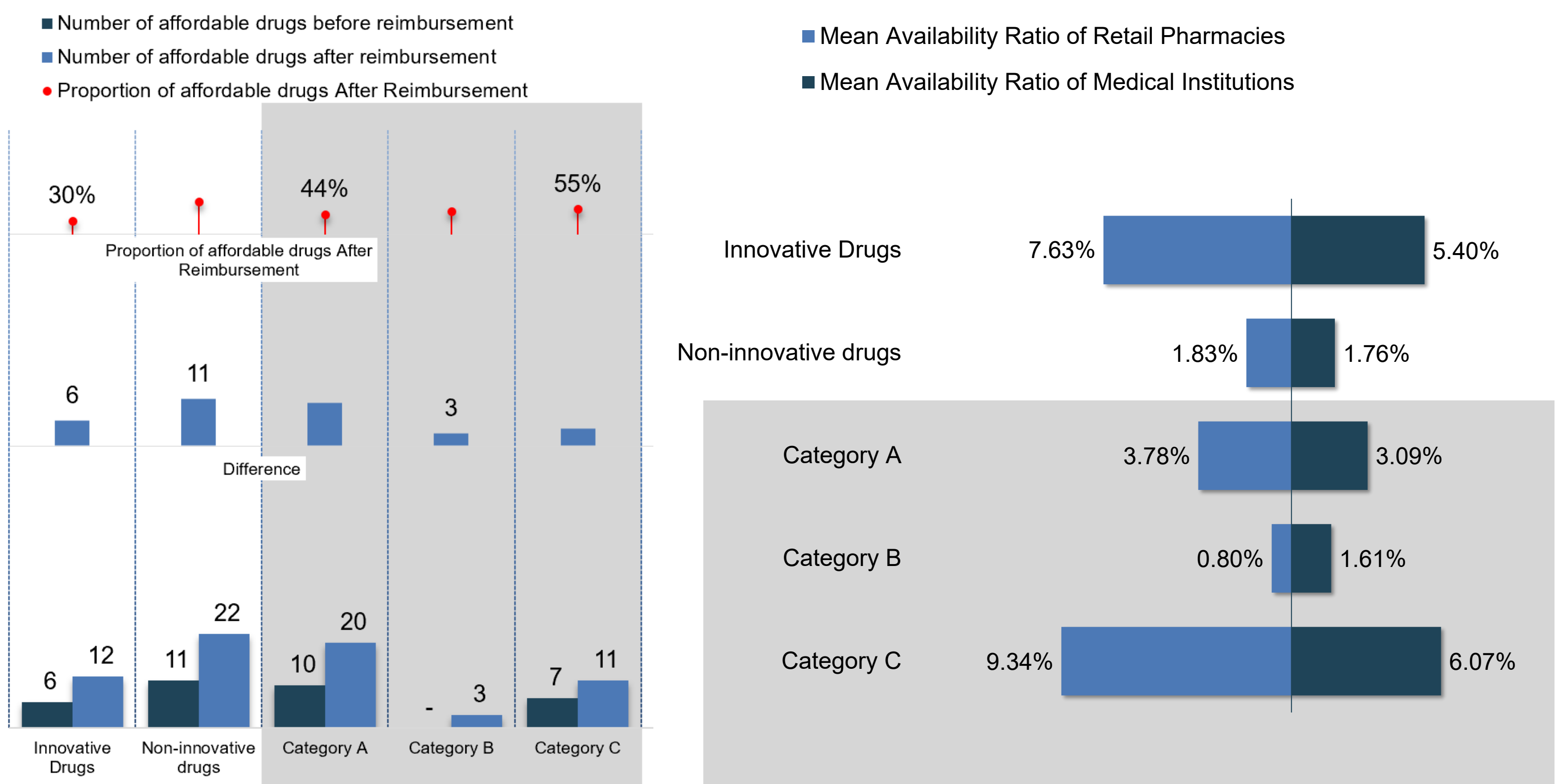
Results-2

Figure 2 Key Characteristics of Included Orphan Drugs



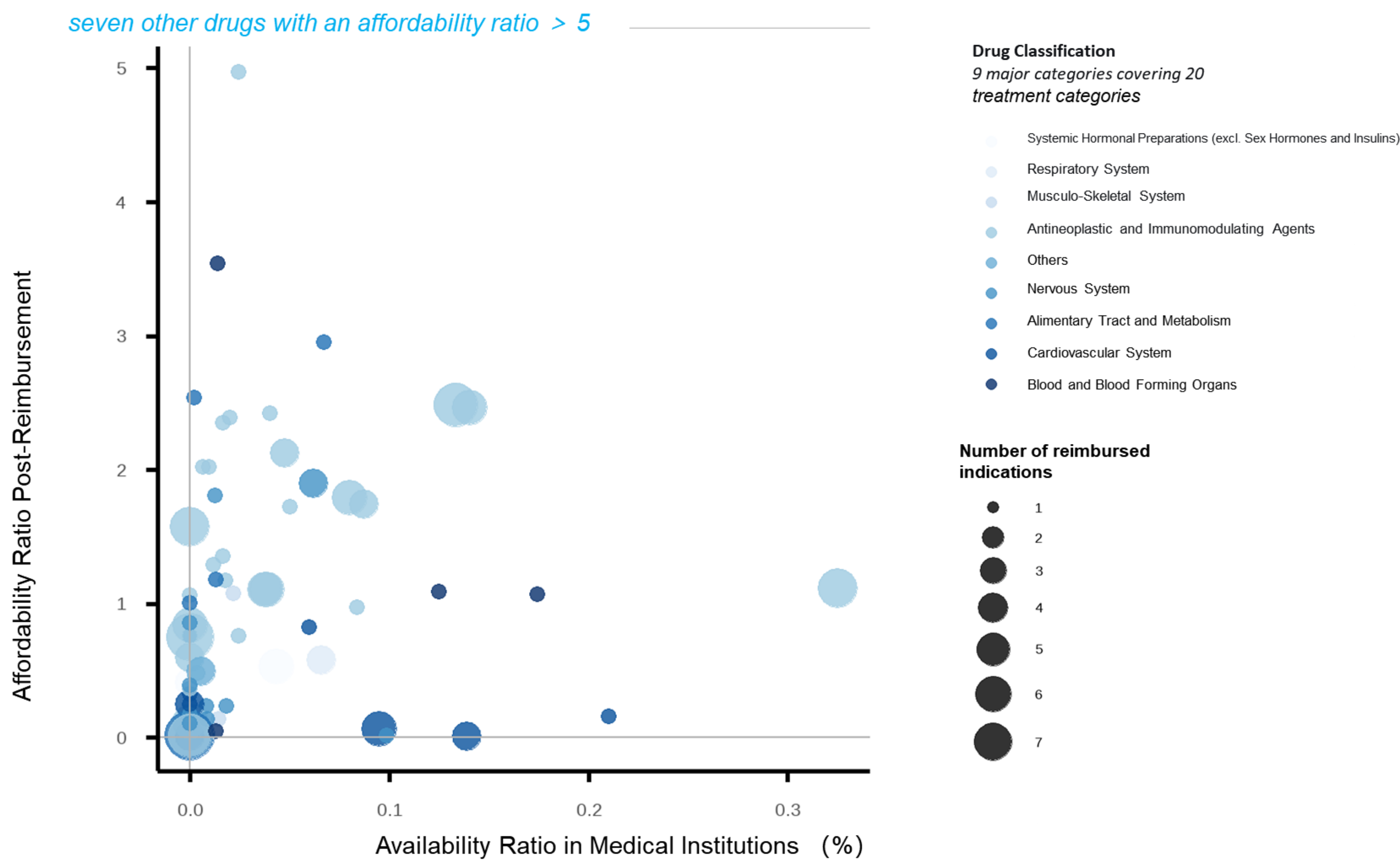
- Multiple sclerosis and idiopathic pulmonary arterial hypertension had the highest number of orphan drugs (7 each).
- Among these listed drugs, 40 (56.34%) were innovative drugs, 17 (23.94%) were biological products, and 20 (28.17%) had both rare and common indications under scope.

Figure 3 Subgroup Analysis on Affordability and Availability



- Hospital and pharmacy availability remained low (3.81% and 5.10%, respectively).
- The number of affordable drugs (the ratio ≤ 1) increased from 17 (23.94%) to 34 (47.89%) after reimbursement.

Figure 4 Cross-Analysis of Affordability vs. Availability of Orphan drugs (Medical Institutions)



- Orphan drugs of different drug classifications currently show differences in accessibility.

Conclusion

The NDPN mechanism accelerated rare disease drug inclusion, reduced prices, and improved accessibility. However, institutional availability gaps and affordability disparities for innovative drugs persist.

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Data Updated as of July 2025