

Title (Title Case): Application Status and Standardized Pathways of Real-World Data in Reimbursement Access for Rare Disease Drugs in China

Authors (Last Name, First Initial): Tao X, Zhang J, Li J, Yang Y, Mao N, Li J

Affiliation: School of International Pharmaceutical Business, China Pharmaceutical University, Nanjing, Jiangsu, China

Abstract

OBJECTIVES: Rare disease drugs face persistent evidence gaps in China because small patient populations, disease heterogeneity, and ethical constraints limit conventional randomized trials. This study assessed how real-world data (RWD) are currently used in national reimbursement access for rare disease drugs in China and proposed a standardized pathway for generating decision-grade real-world evidence (RWE).

METHODS: We identified rare disease drugs included in China's National Reimbursement Drug List from 2021 to 2025 and linked them to public review reports from the Center for Drug Evaluation and National Healthcare Security Administration disclosure materials. We extracted approval pathway, pivotal study design, and RWD submission status. We also reviewed policy frameworks and technical practices in the United States, United Kingdom, and Japan to summarize requirements for study design, data governance, statistical analysis, and policy alignment.

RESULTS: Fifty-six rare disease drugs were included during 2021-2025. Among 32 drugs with complete review and reimbursement disclosure information, pivotal evidence was mainly derived from placebo-controlled randomized trials (59%) or single-arm studies (19%), and these data remained the core effectiveness evidence for reimbursement assessment. RWD were submitted for 53% of drugs but were used mainly for postmarketing safety and effectiveness validation rather than as key clinical evidence for initial reimbursement decisions. International experience indicates that RWD can support external controls, long-term effectiveness and safety monitoring, cost-effectiveness analysis, risk-sharing agreements, and postaccess reassessment when data relevance, completeness, accuracy, transparency, and regulatory compliance are assured.

CONCLUSIONS: China has established an emerging institutional foundation for RWD use in drug development and regulatory review, but reimbursement-specific application scenarios and appraisal standards remain unclear for rare disease drugs. A national reimbursement-oriented RWD framework is needed to define fit-for-purpose study designs, strengthen linkage of disease registries, clinical care, and claims data, and connect RWE with access appraisal, payment standards, coverage scope, and dynamic risk-sharing mechanisms.