Review

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Health technology assessment and medical insurance access for rare disease drugs in China: A policy review with quantitative insights from publicly available data

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SUMMARY: To ascertain the status and propose optimization strategies for rare disease drugs (RDDs) value assessment in the scenario of the National Reimbursement Drug List (NRDL) dynamic adjustment in China, we conducted a narrative policy review that synthesized published literature and policy documents, supplemented by a secondary descriptive statistical analysis of publicly available 2022–2024 year NRDL negotiation data to contextualize recent reimbursement practices for rare disease drugs in China. This study found that value assessment of RDDs largely aligned with the traditional framework, encompassing five key dimensions: safety, efficacy, economic evaluation, innovation, and equity. Considering disease severity and the competitive landscape, innovative RDDs tend to receive higher clinical value ratings, higher willingness-to-pay thresholds, and broader policy support across the healthcare system. Between 2022 and 2024, a total of 60 RDDs applied for NRDL inclusion, with 43% successfully reimbursed. Most applicants were either original research drugs already approved overseas or modified new drugs launched domestically and abroad. Notably, 42% of the drugs had achieved global first launches before 2015, thereby accumulating extensive clinical evidence, and 58% submitted randomized controlled trial (RCT) data. The proportion of drugs supported by RCT evidence in the reimbursed group was significantly higher than the figure in the non-reimbursed group, whereas the proportion of drugs with pediatric indications were relatively lower in the reimbursed group. No significant differences were observed in other value assessment dimensions between successful and unsuccessful applicants. It is recommended that China develop detailed health technology assessment (HTA) guidelines and real-world evidence (RWE) guidance tailored for RDDs, facilitating the generation of high-quality evidence and decreasing decision-making risks associated with the value assessment of innovative RDDs.

Keywords: rare disease, health technology assessment, marketing access, medical insurance reimbursement

1. Introduction

Rare diseases impose a substantial burden on patients and their families, and improving the quality of life for individuals living with rare diseases requires concerted public efforts across society. In China, government agencies, academic institutions, charitable organizations, and patient groups have collaboratively advanced the development of a more comprehensive security system for rare diseases. At the national level, the government has continuously strengthened top-level design and provided end-to-end support for the research, market entry, and utilization of innovative rare disease drugs (RDDs). Since 2018, the National Health Commission has released two editions of the National Rare Disease List, covering a total of 207 diseases (*1,2*). In 2024, the State Council Executive Meeting reviewed and approved the Implementation Plan for End-to-End Support of Innovative Drug Development, which reinforced comprehensive policy measures, integrating pricing regulation, reimbursement policies, commercial insurance, drug availability and use, as well as financing mechanisms, while optimizing regulatory review and hospital performance assessment to accelerate the development of innovative medicines. In 2025, the General Office of the State Council issued the Opinions on Deepening the Reform of Drug and Medical Device Supervision to Promote High-Quality Development of the Pharmaceutical Industry, which emphasized accelerating the review and approval of drugs and devices for rare diseases, exempting eligible innovative drugs for rare diseases from clinical trials, and prioritizing review for urgently needed therapies, including cell and gene therapies (*3*). In the same

year, the National Healthcare Security Administration (NHSA) and the National Health Commission (NHC) jointly introduced Measures to Support High-Quality Development of Innovative Drugs, which explicitly provided end-to-end support for RDDs in priority areas such as R&D, market entry, hospital adoption, and multi-source payment mechanisms (*4*). At the practical level, China has established demonstration zones, including the Boao Lecheng International Medical Tourism Pilot Zone in Hainan, the "Hong Kong–Macao Medicine and Device Connect" policy in the Guangdong–Hong Kong–Macao Greater Bay Area, and the Beijing Tianzhu Rare Disease Drug Security Pilot Zone, further addressing unmet clinical needs for RDDs.

With the accelerated launch of innovative RDDs, particularly cell and gene therapies, the traditional health technology assessment (HTA) framework faces significant challenges (*5*). These challenges mainly arise from immature clinical trial evidence, limited sample sizes, reliance on surrogate endpoints, uncertainty regarding long-term benefits, and extremely high drug prices (*6-10*). To address these challenges, HTA bodies worldwide have adapted their pathways to better accommodate treatments for rare diseases (*11-15*). For example, the National Institute for Health and Care Excellence (NICE) in the United Kingdom has incorporated additional elements of value, such as severity, rarity, equity, unmet need, and innovation. Innovative drugs for rare diseases are eligible for higher willingness-to-pay thresholds, or a severity modifier that increases the weight of quality-adjusted life years (QALYs) for severe conditions (*16,17*). In Germany, the Federal Joint Committee (Gemeinsamer Bundesausschuss, G-BA) assesses rare disease treatments through an orphan medicine pathway that simplifies evidence requirements. To strengthen data collection, France has developed the National Rare Disease Database (Banque Nationale de Données Maladies Rares, BNDMR), while Italy has established multiple monitoring registries to routinely collect product utilization data (*18*).

Unlike other countries, China has yet to establish a separate HTA institution at the national level. Instead, the NHSA directly oversees both purchasing and technical appraisal processes, relying on a network of affiliated research and academic institutions. The HTA of RDDs is primarily conducted by universities and research institutes, and the findings are directly translated into supporting evidence for reimbursement applications. To clarify the current status and challenges of value assessment for RDDs, this study reviews and summarizes the value dossiers of RDDs that participated in National Reimbursement Drug List (NRDL) price negotiations between 2022 and 2024.

The plan is to clarify the value assessment elements of RDDs that support reimbursement decisions, identify the challenges encountered in value assessment and reimbursement processes, and propose future optimization pathways by drawing on international experience.

2. Policy review and analysis

2.1. Literature and policy review about HTA-informed NRDL for rare diseases

A narrative review was conducted using the keywords "rare diseases", "orphan drugs", "health technology assessment", "reimbursement access", "National Reimbursement Drug List negotiations", and "comprehensive clinical evaluation of drugs" in PubMed, China National Knowledge Infrastructure (CNKI), and the official website of the NHSA from inception to 1st August, 2025, without limitation on article types. This review identifies publicly available Chinese and English literature and policy documents related to HTA and reimbursement access for RDDs in China, which constitutes the core of the review. We included health or medical insurance policy research related to NRDL price negotiation and HTA of RDDs, aiming to summarize the dimensions and elements of HTA applied to RDDs during the reimbursement process. Studies focusing on a single disease area (*e.g.*, oncology) regarding NRDL negotiation impacts on price or clinical benefits of drugs were excluded.

2.2. Policy document synthesis

Since the establishment of the NHSA in 2018, the NRDL has undergone annual dynamic adjustments in charge of NHSA. NHSA publishes yearly NRDL adjustment work plans, in which the adjustment scope and work procedure were elucidated. The scope of adjustment includes newly approved drugs with generic names, as well as drugs with significant changes in indications or therapeutic functions approved by the National Medical Products Administration (NMPA). The adjustment work procedure consists of five phases: preparation, application, expert evaluation, price negotiation or bidding, and publication of the final results. NHSA published the drug value dossiers submitted by pharmaceutical companies during the application stage and released the updated NRDL after the negotiation or bidding stage. These public disclosures were major sources of policy practice information. Starting in 2022, RDDs approved by the NMPA were listed as a separate category within the NRDL adjustment scope. To avoid bias, this study focuses solely on RDDs that participated in NRDL adjustments between 2022 and 2024 (*19-21*).

2.3. Secondary descriptive quantitative analysis

Based on the list of drugs that passed formal review and the publicly available drug value dossier released

by the NHSA, a secondary descriptive analysis was conducted to provide contextual quantitative support for the narrative review and was not intended to identify determinants, predictors, or causal mechanisms of reimbursement outcomes. Thus, this study analyzed the characterization of the RDDs involved in NRDL adjustments and the current status of their value assessment evidence, with results presented as drug counts and the percentages of each value feature. Drugs were grouped according to reimbursement success, and Pearson's chi-squared test was applied to categorical data for intergroup comparisons to explore drug value features differences between reimbursed group and non-reimbursed group. Two-tailed p values < 0.05 were considered statistically significant. All descriptive analysis were performed using Excel (Microsoft 365) and intergroup comparative analysis were performed using STATA/MP 16 (Stata Corp).

3. Value assessment framework and current status for rare diseases drugs applying for NRDL

3.1. Value domains and key considerations in each domain for rare diseases drugs

The value assessment of RDDs for reimbursement is conducted within the traditional value assessment framework. In addition to basic drug information, the value assessment of drugs primarily encompasses five key dimensions: safety, efficacy, economic evaluation, innovation, and equity, each with distinct connotations (*22*) (see Table 1). These evaluation criteria apply to all drugs seeking reimbursement access. Absolute safety and efficacy, as well as relative safety and efficacy compared with reference drugs, constitute the foundation of drug value assessment and reimbursement decision-making.

Economic evaluation of drugs, in addition to

Table 1. Overview of value assessment domains and items of the National Reimbursement Drug List (NRDL) price negotiation

Domain	Items
Basic information	• Generic name of the drug • Registered formulation • Indications/therapeutic functions as described in the package insert • Dosage and administration • First marketing approval date in mainland China • Current market status of drugs with the same generic name in mainland China • Country/region of the first global marketing approval and the corresponding approval date • Whether the drug is over-the-counter (OTC) • Recommended reference drugs, and comparative advantages and disadvantages relative to reference drugs or other drugs in the same therapeutic area already on the market • Basic information on the treated disease, unmet medical needs addressed, and disease prevalence in mainland China
Safety	• Safety information reported in the drug's package insert • Incidence of adverse reactions for the drug domestically and internationally • Major safety advantages and disadvantages compared with other drugs in the same therapeutic area listed in the NRDL
Efficacy & Effectiveness	• Efficacy advantages and disadvantages of the drug compared with comparator drugs in clinical trials and real-world studies • Recommendations in clinical guidelines or treatment protocols • Description of the drug's efficacy in the Technical Review Report issued by the Center for Drug Evaluation (CDE) of the National Medical Products Administration (NMPA) • Comparative efficacy advantages and disadvantages relative to other drugs in the same therapeutic area listed in the NRDL • Rationale for the formulation of traditional Chinese medicine (TCM) products and relevant descriptions of how the formulation leverages TCM therapeutic benefits
Economy	• Drug sales revenue in mainland China from January 1, 2021, to June 30, 2023 (including all formulations; if not exclusive, focus primarily on the company's own products) • Current pricing and cost information • Projected sales over the next three years • Impact on the health insurance fund • Cost-effectiveness and other economic evaluations
Innovation	• Key innovative features • Efficacy or safety advantages resulting from the innovation • Whether the drug is supported by national major scientific and technological initiatives (*e.g.*, "Major New Drug Creation" projects) • Whether the drug is an innovation with independent intellectual property rights • Drug registration category • (For traditional Chinese medicine products) Degree of heritage or lineage of the formulation
Equity	• Public health impact of the treated disease • Compliance with the "basic insurance coverage" principle • Whether the drug can address gaps in the NRDL • Clinical management complexity and other relevant considerations

cost-effectiveness and budget impact analyses, also considers publicly available drug prices domestically and internationally (including post-charity donation prices) and the historical sales data in mainland China over the past three years. Assessments of innovation and equity are closely linked to Chinese government policies supporting innovative drugs, reflecting the integration of reimbursement policy with regulatory approval and clinical use policies. Factors such as first-in-class innovative drugs, drugs supported by national major scientific and technological initiatives (*e.g.*, "Major New Drug Creation" projects), urgently needed overseas drugs, encouragement for pediatric drug development, and rare diseases with high severity are considered as additional positive factors during the evaluation process.

3.2. Add-on value considerations for rare disease drugs

The specificity of value assessment for RDDs is mainly reflected in both the evaluation content and the preferential weighting given to RDDs during the review process due to the disease severity and highly unmet clinical needs. For example, innovative RDDs tend to receive more policy support from various regulatory authorities, and many drugs listed in the NHC's catalogue of urgently needed medicines are RDDs. Drugs for rare diseases with higher severity often receive higher clinical value scores. In economic evaluations, elevated willingness-to-pay thresholds are applicable to pediatric drugs, RDDs, end-of-life treatments, and first-in-class medicines (*23*). This approach aligns with adjustments made by international HTA bodies for value assessment of rare diseases (*16,17*).

3.3. Various value characteristics of rare disease drugs applied for and reimbursed by national basic healthcare insurance

The success rate of negotiations for RDDs was not high. Between 2022 and 2024, a total of 77 value dossiers were submitted. After excluding duplicate submissions in the same year, 60 RDDs applied for reimbursement, of which fewer than half (43%) were successfully included in the NRDL (see Table 2). Most of the submitted RDDs were exclusive products (n = 51, 85%).

Both older drugs that have been on the market for many years and high-value innovative drugs with a relatively short market history applied for NRDL price negotiation. Fewer than half of the drugs (n = 25, 42%) had their first global marketing approval more than 10 years ago. Regarding innovation, the majority of submitted RDDs were original drugs already marketed abroad or improved new drugs marketed domestically and internationally (n = 33, 55%), followed by generics (n = 21, 35%). Among these generics, some were based on original drugs not yet marketed in China (n = 8, 13%), while innovative drugs not yet marketed either domestically or internationally were rare (n = 6, 10%). This phenomenon was partially because the NRDL dynamically adjusted annually began from 2018, with several older drugs on the market for many years without opportunity to be included in the NRDL before that. Therefore, for RDDs that have been available for many years, the traditional value assessment framework remains applicable. To date, no cell or gene therapies for rare diseases have applied for reimbursement in China. Currently, a gene therapy for Leber's hereditary optic

Table 2. Value characteristics distribution of rare diseases drugs passing the formal review of national reimbursement drug list price negotiation during 2022–2024

Characteristics	Total (n = 60)	Reimbursed (n = 26)	Non-reimbursed (n = 34)	p-value
Exclusive products	51 (85%)	23 (88%)	28 (82%)	0.51*
Drugs with multiple indications	14 (23%)	4 (15%)	10 (29%)	0.20*
Drugs with non-rare disease indications	11 (18%)	3 (12%)	8 (24%)	0.23*
Innovation				0.23*
Innovative drugs not yet marketed domestically or internationally[a]	6 (10%)	5 (19%)	1 (3%)	
Innovative drugs marketed abroad or improved new drugs[b]	33 (55%)	13 (50%)	20 (59%)	
Generics based on original drugs not marketed in China[c]	8 (13%)	3 (12%)	5 (15%)	
Other generics[d]	13 (22%)	5 (19%)	8 (24%)	
Reported randomized controlled trials (RCTs)	35 (58%)	21 (81%)	14 (41%)	< 0.01*
Reported patient-reported outcomes (PROs)	11 (18%)	5 (19%)	6 (18%)	0.88*
Pediatric indications	32 (53%)	10 (38%)	22 (65%)	0.04*
Applications citing a blank reference drug	28 (47%)	9 (35%)	19 (56%)	0.10*
First global marketing approval before 2015	25 (42%)	14 (54%)	11 (32%)	0.09*
Priority review	28 (47%)	13 (50%)	15 (44%)	0.65*
Breakthrough therapy designation	9 (15%)	5 (19%)	4 (12%)	0.42*
Orphan drug designation	20 (33%)	9 (35%)	11 (32%)	0.85*
Listed as urgently needed, encouraged for development, or encouraged for generic submission	21 (35%)	11 (42%)	10 (29%)	0.30*

Note: [a] Innovative drugs not yet marketed domestically or internationally: Chemical drugs, Class 1 ; Therapeutic biologics, Class 1. [b] Innovative drugs marketed abroad or improved new drugs: Chemical drugs, Class 5.1 ; Therapeutic biologics, Class 3.1; Chemical drugs, Class 2.2. [c] Generics based on original drugs not marketed in China: Chemical drugs, Class 3. [d] Other generics: including Chemical drugs, Class 4; Chemical drugs, Class 5.2; Original chemical drugs, Class 5; Original chemical drugs, Class 6. *Pearson's chi-squared.

neuropathy has been approved for priority use in Boao but has not yet received formal approval domestically. With high-value and clinical uncertainty innovative drugs applying for NRDL, the current value assessment framework needs to be adjusted to comprehensively assess the innovative drugs.

Multiple indication RDDs drove the value assessment framework adjustment. About 23% of drugs were indicated for multiple conditions, of which 79% (n = 11) had non-rare disease indications. Typically, the main indication should be designed for multi-indication drugs to assess drug value and form the basic medical insurance payment price, with drug indication for rare diseases as an add-value factor.

Clinical evidence quality raises decision-makers' and reviewers' concerns. Fifty-three percent of the drugs had pediatric indications. Forty-seven percent of applications cited a blank reference drug, and 58% submitted randomized controlled trial (RCT) data, while patient-reported outcomes (PROs) were infrequently reported (n = 11, 18%).

Full-chain policy supports drug authority and clinical rational use for RDDs. In terms of policy support, approximately half (47%) of the drugs received priority review, 15% were designated as breakthrough therapies, 33% were granted orphan drug status, and 35% were included in the National Health Commission's lists of urgently needed, encouraged for development, or encouraged for generic submission drugs.

Among the RDDs, higher submission/reimbursement frequencies were observed for multiple sclerosis (4 submitted, 3 reimbursed), myasthenia gravis (4; 2), pulmonary arterial hypertension (3; 2), and neuromyelitis optica (2; 2) (see Figure 1 and Figure 2). The reimbursed RDDs and corresponding indications are shown in Table 3.

3.4. Comparisons of value characteristics between reimbursed and non-reimbursed drugs groups

Clinical value remains the major factor for reimbursement of RDDs. The proportion of RDDs with RCT evidence in the reimbursed group was significantly higher (81% *vs.* 41%, $p < 0.01$). The proportion of drugs with pediatric indications was higher in the reimbursement failure group (65% *vs.* 38%, $p < 0.05$). No significant differences were observed between the success and failure groups in terms of exclusivity, first global approval date, drug innovation, multiple indications, blank reference drug application, PRO submission, or policy support (see Table 2). These findings provide contextual quantitative insight for HTA of RDDs applying NRDL pricing negotiation.

3.5. Economic evaluation of RDDs requires more detailed guidance

Currently, reimbursement assessments consider elements such as treatment course costs, historical and projected sales, budget impact, and cost-effectiveness. Model-based cost-effectiveness analyses are optional. According to China's pharmacoeconomic evaluation guidelines, the discount rate applied in economic models for RDDs is the same as for other drugs, with both costs and outcomes discounted at 5%. By contrast, NICE allows lower discount rates (1.5%) for drugs with long-term patient benefits (*16*). With the anticipated market entry of one-time, lifelong-benefit therapies such as cell and gene therapies, China will need to issue more detailed HTA guidelines specifically for rare disease drugs (*22*).

4. Moving forward

4.1. Exploring real-world evidence (RWE) applications and standards for RDDs

The application scenarios and standards for RWE of RDDs are being explored, particularly for innovative RDDs granted conditional approval based on single-arm clinical trial data due to immature trial evidence. Historical data indicate that statistical differences of the proportion of drugs with RCT evidence between reimbursement and non-reimbursement group were noted. In 2025, national policymakers issued guidance encouraging the use of RWE to support the inclusion of innovative drugs in the NRDL, as well as their renewal and adjustment of reimbursement coverage. However, quality standards and specific application scenarios for RWE have not yet been publicly defined (*24*). Currently, the International Society for Pharmacoepidemiology (ISPE) working group, in collaboration with HTA experts, has developed a bias assessment tool for real-world studies of drug safety and effectiveness. Potential for bias is assessed across three domains: *i*) bias due to study design (including time-related bias, inappropriate adjustment for causal intermediaries, depletion of outcome-susceptible individuals, reverse causation, detection bias, and informative censoring), *ii*) misclassification bias (exposure, outcome), and *iii*) bias due to confounding (residual confounding) (*25*). Innovative RDDs already used in demonstration zones have accumulated clinical data, with 42% of drugs having more than ten years of clinical use. Therefore, it is recommended that China establish RWE quality standards for RDDs, drawing on international experience, to provide evidence for drug safety and effectiveness in real-world clinical settings, as well as data on dosage and treatment duration, thereby supporting value assessment of RDDs.

4.2. Building rare disease data systems to support value assessment

Immature clinical evidence for innovative RDDs and unclear disease trajectories pose high decision-making

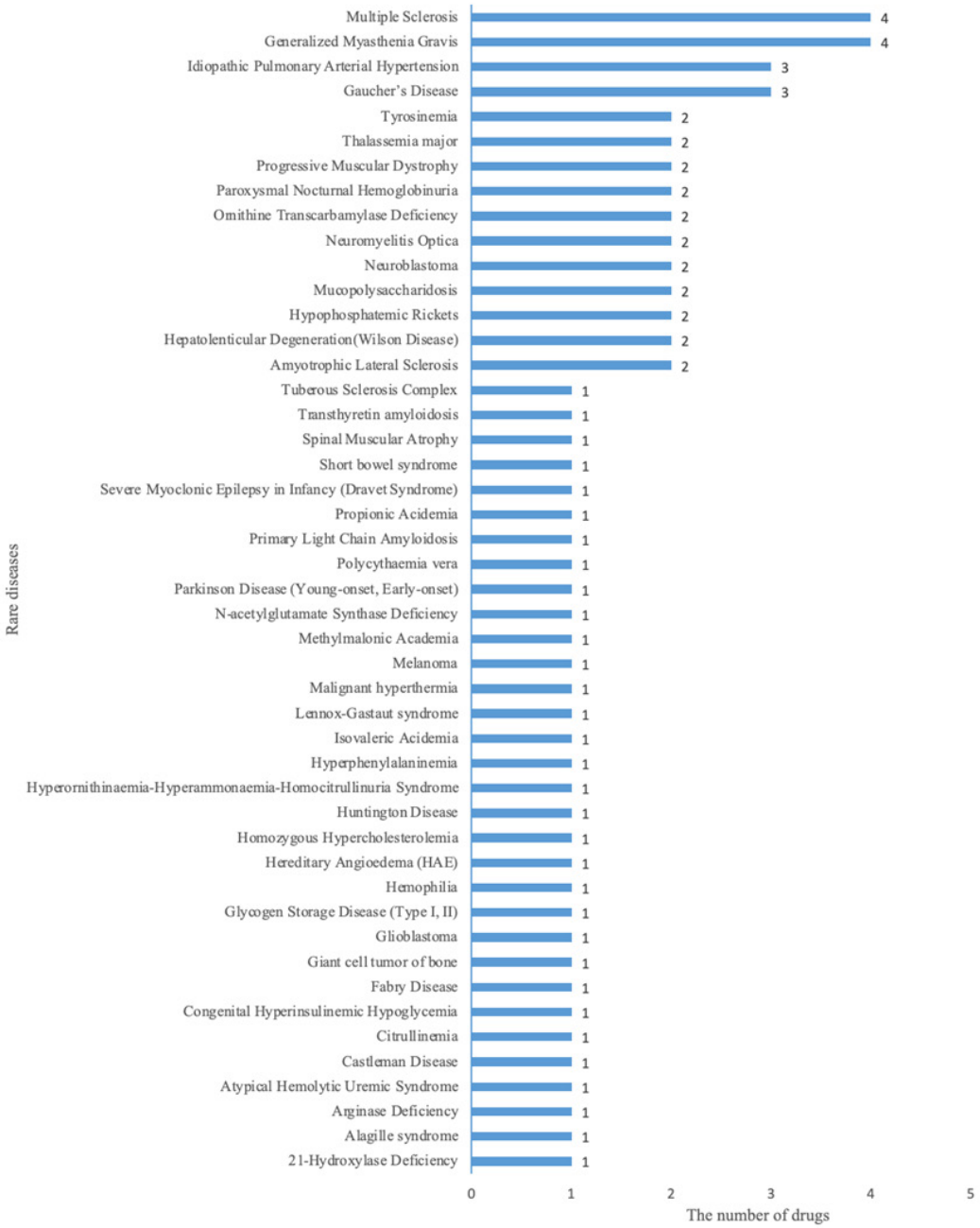

Figure 1. Distribution of drug indications among those drugs applying for national reimbursement drug list price negotiation during 2022–2024.

risks for reimbursement authorities, clinicians, and patients, making it difficult to evaluate long-term risk-benefit profiles. The China Alliance for Rare Diseases has been actively promoting the development of rare disease databases, including the national rare disease registry system, which integrates patient treatment data, drug data, hospital data, expert data, and disease data. Disease-specific cohorts, such as for achondroplasia, are currently being constructed. These data systems enable epidemiological analysis and disease trajectory mapping, including patient sociodemographic, genotyping, treatment regimens, and outcomes, providing critical support for RDD value assessment.

4.3. Introducing commercial health insurance and innovative payment mechanisms

To address the challenges posed by high-cost innovative therapies, such as cell and gene therapies, international HTA bodies have implemented innovative payment mechanisms, including outcome-based reimbursement, to mitigate the budget impact of high-value drugs (*6,26*). In 2025, the NHSA proposed establishing a commercial health insurance innovative drug catalog, focusing on drugs with high innovation, substantial clinical value, significant patient benefit, and coverage beyond basic health insurance, thereby improving China's multi-tiered

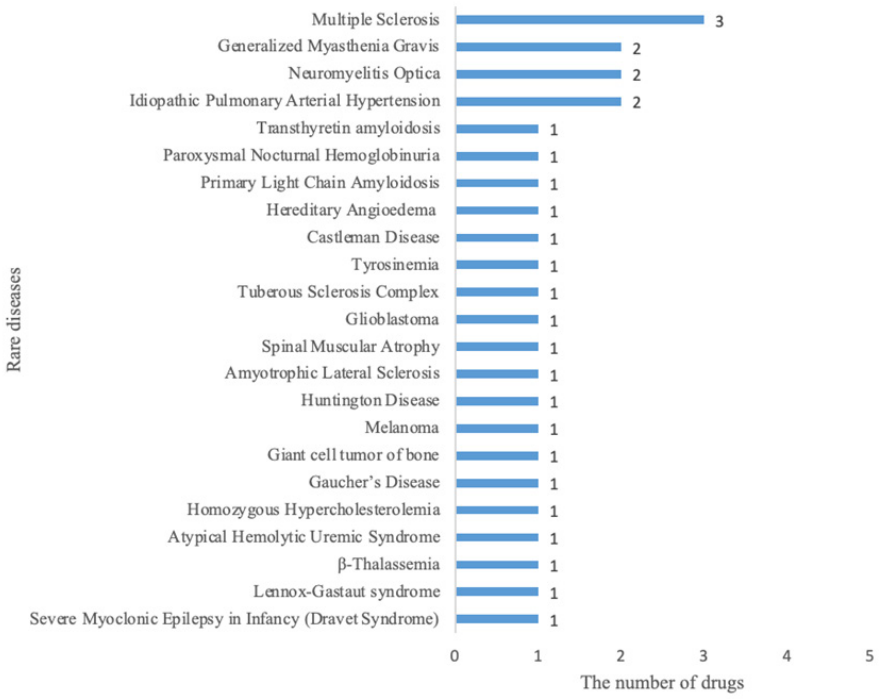

Figure 2. Distribution of drug indications among those drugs listed in national reimbursement drug list *via* price negotiation during 2022–2024.

Table 3. Rare diseases drugs and corresponding drug indications listed in National Reimbursement Drug List (NRDL) *via* price negotiations during 2022 to 2024.

Drug Generic Name	Indication
Beraprost Sodium Sustained-release Tablets	Idiopathic Pulmonary Arterial Hypertension
Bozitinib Enteric Capsules	Glioblastoma
Daratumumab Injection (Subcutaneous Injection)	Primary Light Chain Amyloidosis
Deferasirox Granules	Thalassemia major
Clobazam Tablets	Lennox-Gastaut syndrome
Tafamidis Meglumine Soft Capsules	Transthyretin amyloidosis
Narlumosbart Injection	Giant cell tumor of bone
Stiripentol For Suspension	Severe Myoclonic Epilepsy in Infancy (Dravet Syndrome)
Tunlametinib Capsules	Melanoma
Ezetimibe and Atorvastatin Calcium Tablets (II)	Homozygous Hypercholesterolemia
efgartigimod alfa-fcab injection	Generalized Myasthenia Gravis
Tetrabenazine Tablets	Huntington Disease
eliglustat tartrate capsules	Gaucher's Disease
Nitisinone Capsules	Tyrosinemia
Satralizumab Injection	Neuromyelitis Optica
Sirolimus Gel	Tuberous Sclerosis Complex
Ozanimod Hydrochloride Capsules	Multiple Sclerosis
Eculizumab Injection	Paroxysmal Nocturnal Hemoglobinuria; Atypical Hemolytic Uremic Syndrome; Generalized Myasthenia Gravis
Siltuximab for Injection	Castleman Disease
Ofatumumab Injection	Multiple Sclerosis
Dimethyl Fumarate Delayed-release Capsules	Multiple Sclerosis
Lanadelumab Injection	Hereditary Angioedema (HAE)
Riluzole Oral Suspension	Amyotrophic Lateral Sclerosis
Risdiplam Powder for Oral Solution	Spinal Muscular Atrophy
Treprostinil Injection	Idiopathic Pulmonary Arterial Hypertension
Inebilizumab Injection	Neuromyelitis Optica

Data source: This table was compiled based on the annal lists of declared medicines that passed form review, along with and updated NRDL from 2022 to 2024 published on the National Healthcare Security Administration website.

healthcare security system.

4.4. Encouraging patient engagement in HTA

Patient engagement is becoming increasingly relevant in regulatory decision-making and HTA for rare diseases (*27-29*). On one hand, it provides insight into the disease and patient needs; on the other hand, traditional cost-effectiveness models often fail to capture the full impact of these conditions. Between 2022 and 2024, only 18% of drugs reported PROs, indicating that the patient voice has not yet been formally incorporated into reimbursement decisions. However, China's drug evaluation authorities have issued guidance outlining how to collect patient experience data throughout the drug lifecycle — including the research planning stage, pre-clinical trial stage, pre-key study stage, pre-marketing application/ marketing application stage, and post-marketing stage — how to apply these data in drug development, and how to implement clinical trials that account for patient experience (*30*). With the integration of regulatory review and HTA reimbursement evidence, HTA agencies may consider incorporating the patient perspective more systematically during reimbursement evaluation, such as patients' previous treatment experiences, disease impact on daily life, and patient-reported quality of life (*31*).

5. Limitations of this review

This study is limited to RDDs that applied for reimbursement between 2022 and 2024. Information on drugs undergoing formal review for NRDL inclusion in 2018–2019 was not published on the NHSA website, and the formal review data for 2020–2021 did not separately identify RDDs. Therefore, only RDDs from 2022 to 2024 were included in this study. Since the submitted drug dossiers did not change during this period, this limitation does not affect the analysis of value assessment elements and the overall evidence profile of RDDs.

Furthermore, in the empirical analysis of reimbursement outcomes, this study did not summarize or analyze economic evidence of reimbursed drugs. This limitation is primarily due to the lack of publicly available information. The NHSA has not disclosed the complete value assessment dossiers or expert review reports submitted by companies after comprehensive review. Consequently, it is not possible to determine whether RDDs that passed comprehensive review included model-based cost-effectiveness analyses. However, based on publicly available information, the main evaluation criteria for economic assessment of RDDs — such as treatment course costs, cost-effectiveness, and budget impact — can be identified.

6. Conclusions

In China, the HTA of RDDs should integrate both traditional and innovative assessment frameworks. To better support reimbursement decisions, the traditional assessment framework is sufficient to demonstrate the clinical, economic, and social value of RDDs that have been marketed internationally for many years or face well-established competitive landscapes. For high-cost innovative RDDs with uncertain long-term clinical benefits, the reimbursement value assessment process should explore incorporation of additional elements, including disease severity, real-world evidence, patient involvement, and innovative payment models, to address uncertainties in value assessment and mitigate associated decision-making risks.

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