

The "China model" for rare disease governance: Policy framework, local implementation, and pathways for optimization

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SUMMARY: Globally, the prevention and treatment of rare diseases is still constrained by limited diagnostic and therapeutic capacity, restricted drug accessibility, and disparities in medical security systems. In response, China has developed a distinct "China Model" of rare disease governance, characterized by national policy leadership and coordinated local implementation. This study systematically reviews policies issued between 2009 and 2026 and it analyzes five domains: *i*) prevention and screening, *ii*) list-based governance, *iii*) clinical diagnosis and treatment systems, *iv*) drug accessibility, and *v*) payment guarantees. Shandong Province is examined as a representative case. Findings show that the central government has established unified standards through two nationally endorsed rare disease lists covering 207 conditions, supported by clinical guidelines and a national collaborative network for diagnosis and treatment of those diseases. Regulatory incentives for drug review and approval have facilitated the inclusion of 126 treatments for patients with rare diseases in the National Basic Medical Insurance reimbursement list, forming an integrated policy framework spanning identification, diagnosis, treatment, and financial protection. At the provincial level, Shandong is aligned with national directives by integrating its case registration system with the national platform, enhancing quality control across its clinical network and developing a multilevel payment mechanism. The core of the "China Model" is the enhancement of clinical capacity through standardized systems and networked organizations, combined with multilevel risk-sharing mechanisms. However, governance challenges persist, including weak inter-organizational policy coordination, barriers to drug accessibility, fragmented coverage schemes, and an underdeveloped data governance infrastructure. Addressing these challenges requires enhanced end-to-end policy implementation and institution of effective local practices at the national level.

Keywords: rare diseases, China model, collaborative network for diagnosis and treatment, case registration, drug accessibility, medical security

1. Introduction

Rare diseases are a heterogeneous group of conditions characterized by a low prevalence, multisystem involvement, and high risks of disability and mortality. More than 7,000 rare diseases have been identified globally. Patients with rare diseases face four interrelated challenges: delayed or inaccurate diagnosis, limited therapeutic options, high treatment costs, and substantial long-term caregiving burdens (1,2). In China, approximately 20 million individuals are living with rare diseases, with approximately 200,000 new cases reported annually. This trend places sustained pressure on healthcare capacity, drug accessibility, and the sustainability of multilevel medical security systems (3).

In response, China has developed a distinct "China Model" of rare disease governance, characterized by national policy leadership and provincial implementation. Shandong Province provides a representative case of institutional evolution. As a populous province with strong healthcare capacity, Shandong has implemented several reforms, including the "4+X" newborn screening model, a provincial clinical collaborative network, a mandatory case registration system, and a drug reimbursement mechanism under the critical illness insurance scheme. These initiatives support evidence-informed governance. This study reviews national policy development (2009–2026) alongside Shandong's experience with its implementation, it examines the structural framework of rare disease governance, and it

analyzes how subnational actors adjust administrative mandates and resource allocation to overcome persistent barriers in "last-mile service delivery."

2. National policy framework: A five-pillar institutional architecture

At the national level, China has established a policy framework with five pillars: prevention and screening; list-based governance; clinical diagnosis and treatment systems; drug accessibility; and financial protection. This framework spans the full continuum from disease identification to health outcomes. It standardizes governance through a national rare disease list, regulates service delivery *via* clinical guidelines and coordinated care networks, enables surveillance through mandatory case registration, aligns incentives across the drug development-approval-reimbursement continuum, and reduces financial risk through a multilevel medical insurance system. The overall framework and operational logic of the system are shown in Figure 1.

2.1. Prevention and screening: Embedded entry points and fragile links

Within China's three-tier system to prevent birth defects, detection of rare diseases is integrated into maternal and child health (MCH) care. The 2017 amendment of the Maternal and Infant Healthcare Law and the 2023 regulations implementing that law incorporated premarital examinations, prenatal diagnosis, diagnosis of genetic disorders, and newborn screening in the statutory scope of MCH care, thereby formalizing risk identification and health education within an existing infrastructure (4,5). The 2019 revision of the

Administrative Measures on Prenatal Diagnosis, the 2009 Administrative Measures on Newborn Disease Screening, and related technical standards established a combined "regulatory framework + technical specifications" system that defines screening protocols, institutional accreditation, and quality assurance requirements (6-8).

This integrated approach enables broad population coverage and efficient incorporation into routine clinical workflows, functioning as a "lead generation entry point" for rare disease identification. However, several limitations remain. The scope of conditions included in routine screening only partially aligns with the national Rare Disease List. In addition, post-screening processes - such as confirmation of a diagnosis, coordination of referrals, case registration, follow-up, and financial coverage - remain inconsistent across regions in terms of specificity, implementation, and interoperability. Consequently, initial screening "leads" do not consistently translate into standardized care pathways. Recent policies, including the Plan to Enhance Birth Defect Detection Capacity (2023–2027) and updated quality control indicators, emphasize enhancing the "diagnosis-and-intervention" phase after positive screening results (9,10). These measures aim to operationalize the full continuum from screening to diagnosis, treatment, and rehabilitation, with particular emphasis on enhancing the "smooth transition" from detection to clinical management.

2.2. List governance: Defining boundaries, setting priorities, and establishing a shared language

In the absence of comprehensive epidemiological data and a unified prevalence threshold, China has adopted

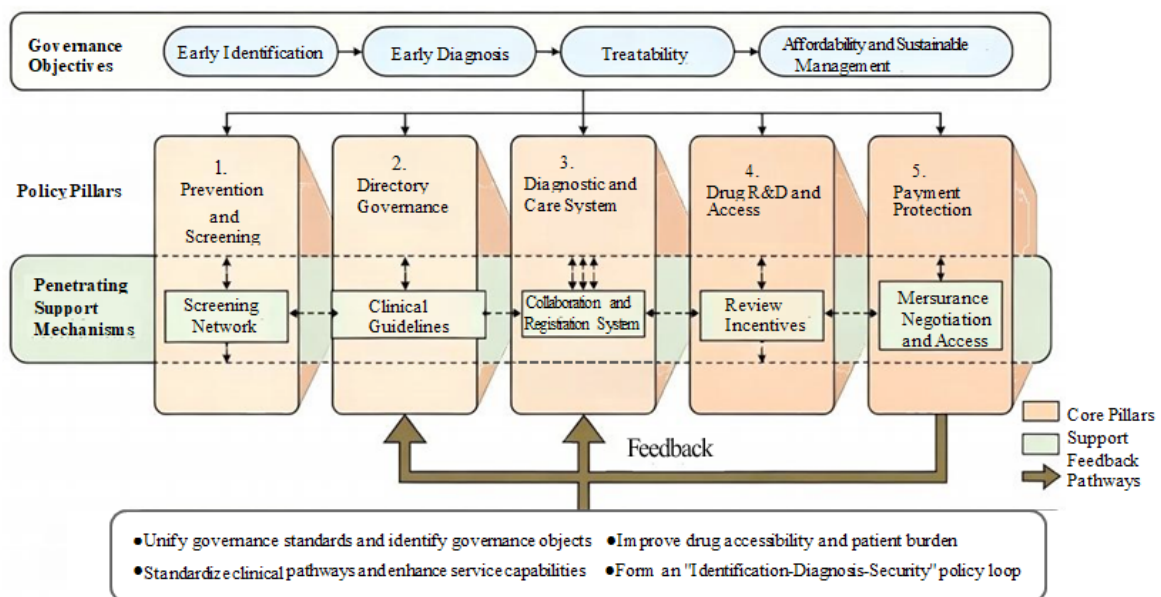


Figure 1. Overall framework and operational logic of China's rare disease governance model.

a "disease list"-based approach to define the scope of rare disease governance. The first national list, issued in 2018, included 121 diseases; the second, issued in 2023, added 86, bringing the total to 207. This list functions as a cross-departmental "common reference framework" for policy coordination (11,12). Its institutional value is reflected in two dimensions. First, it defines governance boundaries and priorities, enabling alignment among clinical guidelines, care networks, case registration systems, drug approval processes, and reimbursement policies. Second, it provides subnational authorities with a standardized "language system" framework that reduces transaction costs in policy implementation.

However, list-based governance must deal with two key sources of tension. The first involves balancing regulatory consistency with the need for timely updates based on emerging clinical evidence. The second concerns strengthening the link between the list as a "definitional tool" and as a "catalyst for patient-level entitlements." Only when the list is systematically integrated into clinical standards, registration systems, drug approval criteria, and reimbursement policies can it evolve from a "unified reference point" into an actionable, patient-centered governance tool.

2.3. Diagnosis and care system: An integrated institutional architecture of guidelines, networks, and registration

To address persistent bottlenecks, including misdiagnosis and delayed diagnosis, China has implemented a tripartite institutional strategy consisting of clinical guidelines, a collaborative care network, and a mandatory case registration system. This approach enhances supply-side capacity for rare disease care. At the normative level, the Guidelines for the Diagnosis and Treatment of Rare Diseases (2019) established standardized pathways for the 121 initial conditions, while the 2025 update extended coverage to 86 additional diseases, achieving full alignment with the expanded list (13,14).

At the organizational level, the National Collaborative Network for the Diagnosis and Treatment of Rare Diseases - composed of national- and provincial-level leading hospitals and affiliated institutions - supports centralized diagnosis, bidirectional referrals, and sharing of resources. Together with the National Rare Disease Medical Centers, it forms a tiered system characterized by "center-led guidance and network-enabled collaboration" (15,16). At the level of data, the mandatory case registration system aggregates standardized clinical data, enabling case management, longitudinal follow-up, quality control, and the generation of natural history and real-world evidence (17).

The core logic of this strategy is the coordination of fragmented resources: clinical guidelines standardize care pathways, the collaborative network reduces interinstitutional barriers, and the registration system

establishes a feedback loop for continuous quality improvement. The central challenge is no longer the existence of these tools, but whether data can be effectively used to facilitate quality assurance, train providers, optimize referrals, and allocate resources, thereby completing the "data → governance → improvement" cycle.

2.4. Drug development and access: From accelerated review to predictable returns

The pharmaceutical sector represents a key site of "chain friction" in rare disease governance, driven by high research and development (R&D) uncertainty, unmet clinical need, limited patient populations, and methodological constraints in evidence generation. Even after market authorization, therapies frequently encounter downstream barriers, including limited hospital adoption, inconsistent supply, and misaligned reimbursement mechanisms.

In response, national policy has evolved towards a comprehensive framework integrating "methodological support," "economic incentives," and "expectations of predictable returns." At the methodological level, the National Medical Products Administration has issued technical guidance on drug development, statistical approaches, natural history studies, and decentralized clinical trials. These measures reduce regulatory uncertainty and promote "patient-centered" development models that incorporate patient perspectives throughout the R&D lifecycle. At the regulatory level, innovative therapies qualify for priority review, and the 2020 revision of the Administrative Measures on Drug Registration improved transparency and predictability (18). Economically, preferential value-added tax policies and tariff adjustments reduce production and import costs, improving expected returns (19). In terms of intellectual property policy, the 2026 revision of the Regulations implementing the Drug Administration Law introduces market exclusivity of up to seven years, contingent on commitments to ensure consistent supply. This establishes an institutional linkage of "reliable supply—exclusivity as a reward" (20).

Collectively, these policies aim to provide credible signals that reduce investment risk. However, effectiveness depends on seamless integration across the access pathway: "approval → listing → hospital adoption → reliable supply → physician prescription → insurance reimbursement." Disruptions in any stage may limit access for patients with rare diseases despite regulatory progress upstream.

2.5. Payment protection: Expanding coverage despite structural gaps

China has established a three-tier payment framework consisting of basic medical insurance, critical illness

insurance, and medical assistance. As outlined in the Opinions on Further Reform of the Medical Security System, this "triple-protection" structure aims to enhance risk pooling for catastrophic expenditures and explore targeted mechanisms for rare disease drug coverage (21). Within this framework, coverage has expanded through dynamic updates to the National Reimbursed Drug List (NRDL), price negotiations for high-cost therapies, and active price regulation. By December 2024, 126 drugs for patients with rare diseases were included, covering 68 conditions. Several high-cost therapies have undergone substantial price reductions through national negotiation, improving patient access (3).

Despite these advances, structural gaps remain. First, disparities between listed and unlisted drugs expose patients to high out-of-pocket costs for effective but unreimbursed therapies. Second, long-term sustainability and equity require stronger empirical support. If registration data, real-world evidence, and budget impact analysis are not systematically integrated into policy adjustment processes, regional variations may persist, leading to fragmented and unequal access across jurisdictions.

3. Shandong's implementation pathway: Operationalizing top-level design and establishing an integrated service chain

Within the national policy framework, Shandong Province has instituted a locally operated model by using the MCH system to expand screening and enhance early detection; integrating provincial case registration with the national platform to support collaborative diagnosis and treatment; enhancing standardization through a regional network of collaborative care, designated hospitals, and quality control centers; and ensuring affordability by anchoring coverage in basic medical insurance while incorporating diversified payment mechanisms. Across five interlinked domains - prevention and screening, case registration, diagnosis and treatment, drug accessibility, and payment security - this approach demonstrates how provincial implementation can bridge systemic gaps and establish a closed-loop service model (Table 1).

3.1. Prevention and screening: Localized "entry expansion" and structured referral mechanisms

Building on the national three-tier framework for prevention of birth defects, Shandong has enhanced risk identification and early intervention from pregnancy through the neonatal period *via* provincial initiatives. Management protocols post-screening have been instituted to ensure systematic referral to diagnosis and treatment pathways.

In terms of prenatal screening, a free provincial program launched in 2017 established a "closed loop" of services for "screening-genetic counseling-referral-

follow-up," providing a standardized model for managing high-risk populations, including those at risk for Down syndrome (22). In 2025, the Administrative Measures on Prenatal Diagnostic Techniques formally defined prenatal diagnosis as the assessment of fetal congenital anomalies and inherited disorders. The policy regulates core components, including genetic counseling, imaging, biochemical and immunological testing, cytogenetics, and molecular genetics, as well as specific modalities (*e.g.*, serum screening, non-invasive prenatal testing, and ultrasound). It also establishes institutional accreditation standards, technical protocols, and quality control requirements, enhancing the continuum of "a condition is suspected based on screening → standardized confirmation → timely referral and intervention" (23).

For newborn screening, the province issued the Administrative Measures on Newborn Screening in 2021, mandating screening for four core conditions: phenylketonuria (PKU), congenital hypothyroidism, congenital adrenal hyperplasia, and glucose-6-phosphate dehydrogenase deficiency. The program has been expanded to include tandem mass spectrometry-based screening for inborn errors of metabolism and universal hearing screening, supported by governance and quality assurance mechanisms (24). Shandong applies a differentiated "4+X" model: the "4" denotes the four fully subsidized conditions, while the "X" represents additional conditions selected by prefecture-level cities based on fiscal capacity and disease burden. Some cities have expanded coverage to 36–48 conditions. Although this multilevel financing structure has accelerated expansion, it has also introduced regional disparities in subsidies and cost burdens, highlighting the need for improved equity and fiscal efficiency (25-27).

3.2. List governance and case registration: Enabling collaborative clinical operations through standardized terminology and provincial-level registration

To enhance the identification of cases of rare diseases and improve the efficiency of inter-institutional referrals, Shandong Province has established a provincial registration system centered on terminology standardization—thereby ensuring effective alignment with the national Collaborative Network for the Diagnosis and Treatment of Rare Diseases and its associated registration framework.

In 2010, the Shandong Association for Rare Disease Prevention and Control was founded to advance academic research, physician training, and public education, providing sustained support to enhance clinical capacity in rare disease identification. In 2017, Shandong became the first province in China to implement a provincial-level rare disease case registration system, covering 53 tertiary hospitals and maternal and child healthcare facilities. This initiative laid the foundation for a cross-institutional infrastructure for aggregation of standardized

Table 1. A comparative analysis of the design and implementation of national policies on rare diseases in Shandong Province

Governance phase	National policy instruments	Implementation in Shandong Province	Mechanism of local modification
Prevention and screening	The Newborn Screening Measures (2009) explicitly delineate requirements for screening, diagnosis, referral, follow-up, and quality control; the Program to Enhance Birth Defect Detection Capacity (2023) enhances the integrated "screening-diagnosis-intervention" continuum.	The Shandong Plan to Implement Free Prenatal Screening (2017) and the Regulations for the Implementation of Administrative Measures on Newborn Disease Screening (2021) promote the standardized implementation of prenatal screening, prenatal diagnosis, genetic counseling, and newborn screening.	Converts national front-end identification requirements into provincial-level entry points for the "screening-consultation-referral-intervention" process.
Disease-specific list management	The Notice on the First Installment of the Disease List (2018) and Notice on the Second Installment of the Disease List (2023) jointly established a unified definition of the target population for rare disease management, thereby instituting a standardized list and delineating clear policy boundaries.	Building on this harmonized framework—which encompasses 207 designated rare diseases drawn from both installments of the list—substantial progress has been made in advancing diagnosis and treatment protocols, case registration systems, health insurance coverage, and legislative research.	These national list standards have been operationalized as a common reference language to facilitate consistent internal identification, collaboration, and effective policy implementation across provinces.
Diagnosis and treatment system	The Collaborative Network Notice (2019) established a national collaborative network and referral mechanism; the Diagnosis and Treatment Guidelines (2019/2025) provided standardized diagnostic and treatment pathways.	Established a provincial-level collaborative network for diagnosis and treatment consisting of 53 member hospitals; the Provincial Hospital is designated as the lead institution to establish a provincial quality control center, and the development of multidisciplinary teams (MDTs) and disease-specific outpatient clinics are promoted.	Adapted the national Collaborative Network and Guidelines framework into a provincially implemented, tiered diagnosis and treatment system in the form of "Collaborative Network- Member Hospitals-Quality Control Centers-MDTs."
Case registration system	The Information System Management Plan (2019) mandates that hospitals in the collaborative network implement standardized case registration, systematic data collection, and rigorous quality control measures and establish a unified, interoperable information platform.	The provincial system for registration of cases of rare diseases was launched in 2017, with continuous improvement of the database. Public responses indicate that 87,600 patients and 207 disease categories have been registered.	The national registration system is being transformed into a comprehensive governance support platform, serving as the foundational infrastructure for provincial databases, routine reporting, and statistical analysis.
Drug development and accessibility	The Opinions on Reforming Review and Approval Procedures (2017) encourage innovation; the VAT Notice (2019) provides tax incentives; the Administrative Measures on Drug Registration (2020) improve review rules; and the Regulations for Implementation (2026) establish a priority review pathway and stipulate a maximum period of market exclusivity of seven years.	The Shandong Biomedical Innovation Action Plan (2024) supports research and development, clinical trials, regulatory review services, and medical insurance coverage of innovative drugs for rare diseases while also facilitating the integration of the "dual-channel" pharmaceutical supply system; the R&D Subsidy Notice (2026) provides subsidies for clinical trials of Class I new drugs.	A provincial-level translational value chain – consisting of R&D subsidies, clinical trials, regulatory review and approval, and linkage to medical insurance – shall be established.
Medical security policy	The Opinions on Medical Insurance Reform (2020) established a three-tiered medical security system consisting of basic medical insurance, critical illness insurance, and medical assistance; the Opinions on Dual-channel Mechanism (2021) promoted the alignment of drug supply and payment for negotiated medications.	The newly added orphan drugs will be incorporated into the dual-channel system; outpatient treatment for 10 rare diseases will be covered under a dedicated, separate payment scheme; and financial burdens will be mitigated through coordinated support from critical illness insurance and medical assistance programs.	The national unified reimbursement framework has been refined into province-level integrated medical security – consisting of the dual-channel system, separate outpatient payment, critical illness insurance, and medical assistance.

Note: In the table, both national policies and their implementation in Shandong Province are referred to using the standardized format "document abbreviation + year." Full document titles are provided either in the main text or in the references. For certain implementation practices in Shandong Province—where no single, dedicated regulatory document exists—the descriptions are synthesized from relevant action plans, official departmental responses, or internal working documents.

case data (28). In 2019, 22 medical facilities—including Shandong Provincial Hospital as the lead—joined the National Collaborative Network for the Diagnosis and Treatment of Rare Diseases, successfully integrating the provincial registration platform with the national system. Concurrently, the scope of registered conditions was expanded to encompass all 207 diseases listed across the two officially issued installments of the National Rare Disease List, thereby establishing a unified, interoperable data foundation to support coordination of intra-provincial referrals and evidence-informed optimization of policy (15).

As of the most recent reporting period, the provincial registration system has a total of 83,600 confirmed cases of rare diseases. Of those, 92.68% come from four key clinical departments: endocrinology, neurology, respiratory medicine, and hematology (according to data from the Shandong System for Registration of Cases of Rare Diseases). This not only reflects the clinical characteristic of rare diseases involving multiple systems but also offers clear guidance for subsequent training in targeted identification and management of follow-up by key departments.

3.3. Diagnosis and treatment system: A collaborative network, designated hospitals, and a quality control center promoting standardized care

To improve accessibility and standardization, Shandong has developed an integrated system consisting of a collaborative network, designated hospitals, expert teams, and a provincial quality control center. This model is grounded in multidisciplinary collaboration and structured referral mechanisms.

In 2019, the provincial Collaborative Network for the Diagnosis and Treatment of Rare Diseases was established, incorporating all Grade-3 general hospitals and Grade-3 MCH hospitals to support cross-institutional consultations and tiered referrals (29). In 2021, the Shandong Province Plan for the Prevention and Control of Rare Diseases designated specialized hospitals and established an expert panel to support complex case management (30). In 2022, the province established the first provincial Center for the Quality Control of Medical Care for Rare Diseases, introducing a three-tier coordinated system involving a lead hospital, member hospitals, and downstream institutions. This system supports standard development, training, assessment of competency, and cyclical quality evaluation, thereby advancing standardization. It also promotes multidisciplinary outpatient clinics, improving comprehensive care capacity (31).

At the institutional level, Shandong Provincial Hospital has established 69 multidisciplinary teams, including 25 dedicated rare disease teams, that provide more than 100 interdisciplinary consultations annually. The number of rare disease diagnoses increased from

108 in 2021 to 131 in 2024, demonstrating expanded diagnostic and treatment capacity (according to data from Shandong Province's Center for the Quality Control of Medical Care for Rare Diseases).

3.4. Drug supply and clinical accessibility: Industrial strength despite persistent gaps in R&D capacity and in-hospital availability

Drug accessibility remains affected by cumulative friction along the "access–procurement–entry into hospitals–supply–reimbursement" pathway. In 2024, Shandong issued the Shandong Biomedical Innovation Action Plan, supporting the R&D and translation of innovative drugs through rare disease clinical trials, review services, and the integration with medical insurance and the "dual-channel" system. Shandong has a strong pharmaceutical base, with 5,924 biopharmaceutical enterprises as of 2023; these are concentrated in Jinan, Qingdao, Heze, Yantai, and Zibo and are supported by established manufacturing capacity (32). In the area of rare diseases, however, research and industrial capacity remain limited. Of more than 800 globally approved drugs for rare diseases, only 252 are marketed in China, nearly 60% of which are imported, and only six are domestically developed innovative drugs. Although two drugs have been launched by Shandong-based enterprises, production capacity is limited, and most firms focus on generics, indicating limited capacity for upstream innovation (33,34).

Policy support for rare disease drug and device development remains insufficient. Provincial regulatory frameworks and incentive mechanisms are underdeveloped, and systems supporting collaboration among academia, industry, and clinical practice are incomplete. Market approval represents only the initial stage; sustained access requires coordination across provincial lists, inclusion in hospital formularies, prescribing systems, supply chains, and reimbursement. Currently, supply depends heavily on imports, and local production capacity is limited. Persistent challenges include restricted entry into hospitals, regional shortages, and high prices, which disrupt treatment continuity for patients with rare diseases, particularly in primary care. Moreover, surveillance systems, stockpiling mechanisms, and emergency allocation protocols remain underdeveloped, further limiting availability and diminishing system reliability.

3.5. Payment protection: A multi-tiered financing framework anchored in basic medical insurance—with persistent gaps between listed and unlisted therapies

Recognizing the high cost of drugs for rare diseases and the need for lifelong treatment, Shandong Province has established a multi-tiered payment system anchored in basic medical insurance, reinforced by cost-sharing

mechanisms, and supplemented by diversified financing instruments to improve affordability and treatment continuity.

Basic medical insurance serves as the foundation. Complete alignment with the NRDL is maintained, and all listed drugs for rare diseases are covered. Through national price negotiations, these drugs have been reduced in price by an average of over 60%. Additionally, 35 costly rare diseases are included in the program for outpatient care for chronic and specified diseases, enabling coordinated reimbursement between outpatient and inpatient care (35).

The critical illness insurance scheme functions as a secondary safety net. For high-cost drugs not included in the NRDL, including those for Gaucher disease and Fabry disease, a segmented reimbursement model applies: a deductible of 20,000 RMB; 80% reimbursement for costs between 20,000–400,000 RMB; and 85% reimbursement for costs over 400,000 RMB, with an annual ceiling of 900,000 RMB (36).

Medical assistance provides targeted protection for vulnerable populations. Individuals receiving special hardship allowances or minimum livelihood support are exempt from deductibles, with reimbursement rates of at least 70%. Medicinal foods for patients with PKU are included and reimbursed at 75% (37,38). At the supplementary level, inclusive commercial insurance products have been introduced; by 2024, these covered 11 rare diseases (39).

In 2024, the specified drug program under critical illness insurance covered 155 patients, with total expenditures of 86.84 million RMB (approximately 560,000 RMB per patient). Inclusive commercial insurance disbursed 37.18 million RMB to 384 patients (approximately 100,000 RMB per patient) (according to data from the Medical Security Bureau of Shandong Province). Despite these gains, structural challenges remain: access to certain innovative, unlisted therapies is limited; out-of-pocket costs remain high for many patients; and the long-term sustainability and equity of the financing system require further refinement.

4. Challenges and limitations

Although China has established a "China Model" of rare disease governance characterized by national coordination and local implementation and although Shandong Province has demonstrated notable institutional innovation, several challenges remain. First, there is still inconsistency across the care continuum. Post-screening processes, including diagnosis confirmation, referral, and follow-up, vary across regions in terms of standardization and operations (40), limiting the conversion of early detection of cases ("identification") into standardized clinical management. While case registration systems aggregate data nationally, their use in quality improvement and resource allocation

remains limited. Second, access to treatment remains constrained. Even when drugs are approved or included in reimbursement lists, barriers such as "hospitals that are difficult to enter," variations in regional supply (41), and inconsistent supply reduce effective access in the "last mile." Third, financing structures remain fragmented. Although the affordability of listed drugs has improved, clinically necessary unlisted therapies lack consistent financing mechanisms, resulting in continued high out-of-pocket costs for patients with rare diseases (42–43). Fourth, data governance remains underdeveloped. Variability in hospital information systems, lack of unified data standards, and the sensitivity of genomic data impede data integration and sharing. Balancing data accessibility with privacy protection remains a critical policy challenge.

5. Policy recommendations

Based on these findings, four policy priorities are proposed. First, enhance integration across the "positive results in screening → confirmation of the diagnosis → referral → follow-up" pathway by clarifying responsibilities and standardizing processes. This should include capacity-building initiatives and the use of real-world data to identify system bottlenecks and expand collaborative networks. Second, enhance pharmaceutical supply security through integrated mechanisms, including real-time monitoring, strategic stockpiling, and emergency allocation. Concurrently implement incentives such as market exclusivity to support domestic innovation and increase production capacity. Third, promote precise financing by incorporating real-world evidence in dynamic assessment of drug value and adjustment of reimbursements. Develop targeted payment mechanisms for high-cost, unlisted therapies, aligning reimbursement with clinical value and fiscal sustainability, while diversifying funding sources to reduce the pressure on basic medical insurance. Fourth, establish a unified and secure framework for data governance. This should include standardized data collection and interoperability protocols, adoption of privacy-enhancing technologies (e.g., secure multi-party computation and federated learning); and tiered access to de-identified data. These measures will support clinical research and policy evaluation while ensuring ethical and legal compliance.

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