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Policy lessons regarding medical security for rare diseases in China: Insights from Zhejiang Province through the multiple streams framework

Miao Wang[1], Chunyu Rong[1], Pingyang Wang[2,3,*]

[1] Department of Medical Affairs, Shanghai Children's Hospital, School of Medicine, Shanghai Jiao Tong University, Shanghai, China;
[2] Zhejiang Medical Security Research Association, Hangzhou, China;
[3] Zhejiang Provincial Commercial Supplementary Medical Insurance Committee, Hangzhou, China.

SUMMARY: Rare diseases pose a persistent challenge to healthcare systems worldwide due to their low prevalence, high treatment costs, and rapid emergence of novel therapies. In China, while significant progress has been made through national rare disease lists and medical insurance negotiations, substantial medical security gaps remain at the subnational level. This Policy Forum examines the decade-long evolution of rare disease–specific medical security policies in Zhejiang Province (2015–2025) to draw broader lessons for designing sustainable and equitable coverage mechanisms under centralized insurance systems. Using the multiple streams framework (MSF) as an interpretive lens, this article puts forth three policy arguments. First, medical security for rare diseases cannot rely solely on basic medical insurance (BMI); instead, it requires institutional layering that combines insurance-based pooling, fiscal instruments, and social co-assistance. Second, in highly centralized governance contexts, policy entrepreneurship is predominantly state-embedded, with administrative agencies playing a decisive role in coupling problems, solutions, and political mandates. Third, policy innovation in relation to rare diseases is not a one-time event but an iterative process, in which each reform generates new problem definitions and policy windows. The Zhejiang experience demonstrates that under institutional constraints such as standardized benefit lists, local governments can achieve strategic innovation within the available institutional space by shifting their policy focus from reimbursing costs for "drugs on the Nationally Reimbursed Drug List (NRDL)" to targeted compensation for expenditures for drugs not on the NRDL. This pathway improves treatment affordability while maintaining fiscal sustainability, providing actionable insights for China and healthcare systems in other countries facing similar structural constraints.

Keywords: multiple streams framework, policy window, rare diseases, medical security, policy change, Zhejiang model

1. Introduction

Rare diseases, also known as orphan diseases, refer to illnesses with a very low incidence, small patient populations, and severe clinical manifestations. The definition of rare diseases varies across countries and regions due to differences in national contexts. In the United States, a rare disease is defined as affecting fewer than 200,000 individuals, or with a prevalence of less than 1 in 1,500; in the European Union, a prevalence below 1 in 2,000 is used as the threshold; in Japan, rare diseases are defined as affecting fewer than 50,000 individuals or with a prevalence of less than 1 in 2,500 (*1,2*). In China, the government defines and manages rare diseases through the publication of the first national Rare Disease List (121 conditions) and the Second national Rare Disease List (86 conditions) (*3,4*).

Rare diseases represent a major global public health challenge. Globally, more than 7,000 rare diseases have been identified, approximately 80% of which are caused by genetic factors, and nearly 70% manifest in childhood, cumulatively affecting around 300 million people worldwide (*5,6*). However, fewer than 10% currently have approved treatments or therapies (*7*). The diagnosis of rare diseases is often prolonged and complex, requiring multiple consultations and examinations, and treatment costs are typically high, placing a dual burden on patients of difficulty being diagnosed and expensive medications (*8,9*). In response to these issues, developed countries and regions such as the European Union, Japan, and the United States have successively introduced policies to promote the research and development of

orphan drugs and reduce costs through legislation, financial support, and market incentives while providing a high rate of reimbursement or special medical security funds for patients (*1,10,11*).

In China, there are nearly 20 million patients with rare diseases and over 200,000 new cases annually. At the national level, a series of institutional initiatives have been introduced to improve medical security for rare diseases (*12,13*). However, within the institutional framework of the National Basic Medical Insurance (NBMI) system, a degree of mismatch persists between its policy orientation of providing "basic, broad, and sustainable" coverage and the cost structure of orphan drugs, which are characterized by high unit prices and small patient populations. Considering the financial sustainability of the medical insurance fund, drugs with annual costs exceeding 300,000 RMB for reimbursement or 500,000 RMB for negotiation are typically excluded from Nationally Reimbursed Drug List (NRDL) (*7*). As a result, dozens of approved rare disease drugs remain outside the medical insurance reimbursement system, even though these high-cost medications often represent the only effective treatment option for some patients (*14*).

Given the limitations of the NBMI in covering rare disease expenditures, local policy innovation has become crucial to closing the coverage gap. Compared to national-level arrangements, local governments have greater flexibility to develop supplementary mechanisms based on fiscal capacity, population size, and the availability of medical resources. Since 2015, Zhejiang Province has led China in the establishment of a provincial medical security scheme for rare diseases, characterized by fiscal investment as guidance, medical insurance funds as the core financing source, and social assistance and charitable support as complementary components (*15*). This institutional arrangement has effectively alleviated limited treatment accessibility and the heavy financial burden faced by patients with rare diseases. An analysis of the decade-long evolution of Zhejiang's medical security policies for rare diseases from 2015 to 2025 can provide practical insights to optimize medical security mechanisms for rare diseases in China and healthcare systems in other countries facing similar structural constraints.

2. Analytical perspective: Interpreting policy change through the multiple streams framework (MSF)

The MSF, proposed by John W. Kingdon, is widely used to explain the mechanisms of policy change (*16*). According to this framework, the formation of public policy depends on three relatively independent yet potentially convergent streams: The problem stream, the policy stream, and the political stream. The MSF highlights the role of contextual and institutional factors in driving policy change, offering a conceptual lens to understand the internal logic through which health policies respond to evolving health challenges. It has been widely applied in health policy research and practice (*17-19*).

This article adopts MSF as its core analytical framework, which consists of three core components (Table 1). The problem stream refers to how social problems are identified, defined, and brought to public attention, driven by factors such as significant changes in indicators, focusing events, and feedback on existing policies. The policy stream involves advocacy and bargaining among policy community members (such as experts, bureaucrats, scholars, and interest groups) to promote their ideas or proposals, with policy proposals

Table 1. Core analytical framework based on the multiple streams framework

Stream Name	Stream Connotation	Driving Factors	Examples of Manifestations
Problem stream	Social problems perceived by decision-makers	Systematic indicators	Prevalence rates, cost data, *etc.*
		Focusing events	Extreme individual cases reported by the media
		Policy feedback	Criticism arising from inadequate coverage of existing policies
Policy stream	Various solutions proposed by the policy community	Policy proposals	Competition, combination, and optimization of various medical insurance, civil affairs, and fiscal policies
		Optimization & consolidation	Comprehensive evaluation and optimization of technical feasibility, value acceptability, and budget constraints
Political stream	The political environment independent of specific problems	Political forces	Strategic directions of national political, economic, and social development
		Interest groups	Institutional and behavioral adjustments of entities like medical insurance departments and public hospitals during reforms
		Public mood	Higher public expectations for people's well-being and healthcare

meeting certain criteria being more likely to gain traction. The political stream encompasses the political environment influencing the policy agenda, including political forces, government transitions, interest group activities, and public mood. When the three streams converge at a critical moment and are propelled by a policy entrepreneur (an individual or group with the resources and willingness to push a specific solution), policy change can be facilitated (*20*).

When this theory is applied to the context of Chinese governance, a notable feature is the localized reconstitution of the role of the policy entrepreneur. Unlike the conventional depiction of policy entrepreneurs as actors operating outside the institutional system, in China they are more commonly administrative agencies within the bureaucratic structure and their key decision-makers. They simultaneously perform multiple functions—including problem definition, policy design, and political coordination—and therefore have greater capacity for policy mobilization and implementation. In Zhejiang Province, relevant authorities leveraged bureaucratic authority and resource mobilization capacity to organically align the specific policy issue of medical security mechanisms for rare diseases with national strategies such as "targeted alleviation of poverty " and "common prosperity," thereby enhancing the political legitimacy and practical feasibility of institutional innovation and facilitating the opening of a policy window.

Medical security policies for rare diseases in Zhejiang were not developed overnight but progressed through three stages—a "special fiscal subsidy," a "special fund," and "multi-actor co-governance"—with each exhibiting distinct stage-specific characteristics. Each reform cycle alleviated existing problems while simultaneously exposing new institutional boundaries and financial pressures, thereby reshaping the problem stream and creating conditions for the opening of the next policy window (Figure 1).

3. Policies regarding medical security for rare diseases in Zhejiang Province

3.1. From fiscal subsidies to institutionalized risk pooling

Between 2015 and 2016, Zhejiang Province explored medical security for rare diseases institutionally, with policy formation reflecting the cumulative effect of the "problem stream." As high-cost orphan drugs successively came to market, the limitations of the existing basic medical insurance (BMI) system in addressing extremely high-cost individual situations became increasingly evident, leaving some patients' families facing substantial financial burdens. At the same time, sustained media coverage of "illness-induced poverty" among families affected by conditions such as Gaucher disease, amyotrophic lateral sclerosis (ALS), and phenylketonuria (PKU) further highlighted institutional shortcomings. Under the combined influence of these factors, rare diseases evolved from isolated clinical challenges into a public policy issue of broader social significance.

At the level of the "policy stream," the policy community—including experts from the medical insurance and healthcare sectors—began to explore solutions aimed at mitigating the financial risks associated with high-cost medications without undermining the

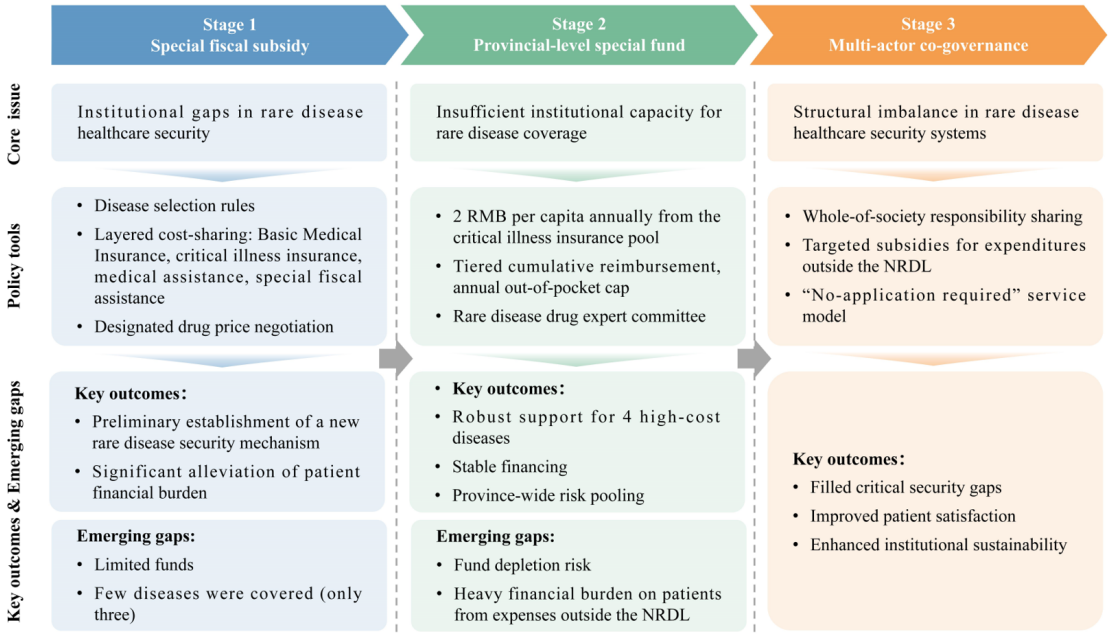

Figure 1. Policy evolution pathways of medical security for rare diseases in Zhejiang Province. *Abbreviation:* NRDL, Nationally Reimbursed Drug List.

foundational framework of the BMI system. Through iterative deliberation, a policy toolkit gradually took shape, centered on "special fiscal subsidies" and complemented by a "layered cost-sharing" mechanism.

In the "political stream," national strategies such as targeted alleviation of poverty and the development of a multi-tiered medical security system provided both institutional legitimacy and policy space. Zhejiang Province's tradition of piloting reforms and its relatively strong fiscal capacity further created favorable conditions for local policy innovation.

Through the interaction of the three streams, a policy window opened. In early 2015, four departments of Zhejiang Province—the Department of Human Resources and Social Security, the Department of Civil Affairs, the former Health and Family Planning Commission, and the Department of Finance—jointly issued the Notice on Enhancing Medical Security for Rare Diseases (Zhejiang Human Resources and Social Security Document No. 126 (2015)) (*21*), the Official Reply on Including Three Special Drugs for Rare Diseases, Including Cerezyme, in Reimbursement under Critical Illness Insurance (Zhejiang Human Resources and Social Security Letter No. 128 (2015)), and the Notice on Issues Concerning the Implementation of Medical Assistance (Special Assistance) for Rare Diseases (Zhejiang Civil Assistance Document No. 36 (2016)) (*22*). The policies were officially implemented across the province on January 1, 2016, marking the first step in exploring the establishment of a special medical security mechanism for rare diseases. With high-cost, case-specific drugs as an entry point, the policy established a "layered cost-sharing" model. After sequential reimbursement by BMI, critical illness insurance, and medical assistance, fiscal special subsidies were introduced to cover the remaining exceptionally high expenses. At the same time, targeted drug price negotiations significantly reduced the costs of treatments for Gaucher disease, PKU, and ALS.

In terms of implementation outcomes, the province treated a total of 365 patients suffering from the three rare diseases mentioned earlier between 2016 and 2018, incurring total drug expenses of 47.84 million RMB. Under the multi-tiered cost-sharing mechanism, BMI covered 2.89 million RMB (with riluzole for ALS included in the NRDL in 2017), critical illness insurance paid 19.67 million RMB, and special fiscal assistance contributed 22.92 million RMB. Together, these three mechanisms absorbed the vast majority of the financial risk, reducing the aggregate out-of-pocket burden for patients to 2.36 million RMB, with the average individual payment declining to approximately 6,400 RMB, thereby substantially alleviating the economic burden of high-cost medical care.

3.2. The emergence of provincial-level special funds

Following the publication of the first national list of Rare Diseases in 2018, rare diseases were formally recognized at the national level, and public concern over the issue of "having available treatments but lacking financial access" increased significantly. In the interim, the limitations of Zhejiang Province's earlier medical security mechanism—primarily reliant on special fiscal assistance—gradually became evident in terms of coverage and financial sustainability, generating notable policy feedback.

Against the backdrop of a continuously intensifying problem stream, the policy community, building on a systematic reflection of prior implementation experience, began to seek more stable and institutionalized solutions. This process led to the formulation of a scheme centered on a "provincial-level special fund."

The maturation of the "political stream" created a critical opportunity to improve medical security for rare diseases. In 2018, the establishment of the Zhejiang Provincial Healthcare Security Administration facilitated the integration of medical insurance management. In 2019, the central government explicitly called for local governments to explore mechanisms to ensure access to rare disease medications, thereby providing policy authorization for local innovation (*23*). Under the combined influence of top-level policy design and institutional restructuring, the problem, policy, and political streams converged once again, opening a new policy window.

In December 2019, the Zhejiang Provincial Healthcare Security Administration, in collaboration with the Provincial Department of Finance, the Health Commission, and the Department of Civil Affairs, jointly issued the Notice on Establishment of a Medical Security Mechanism for Drugs to Treat Rare Diseases in Zhejiang Province (*15*). This policy established the first provincially pooled special fund to cover drugs for rare diseases in China. A dedicated sub-account was created within the provincial medical insurance account, and it operated under separate accounting practices and independent management. Based on the number of participants in the BMI scheme, a fixed annual contribution of 2 RMB per capita was uniformly transferred from the critical illness insurance fund, thereby forming a stable and predictable financing source. In addition, a set of refined management mechanisms was introduced, including a tiered reimbursement system based on cumulative expenditures, an annual cap on individual out-of-pocket payments (set at 100,000 RMB per patient), and the involvement of an expert committee in decisions regarding drug inclusion and evaluation. These measures were designed to ensure both equity and financial sustainability. Moreover, medical assistance programs were implemented in parallel for eligible low-income patients; for those still experiencing financial hardship despite that assistance, additional support was provided by charitable organizations such as the provincial charity federation.

Since its implementation in 2020, fund coverage has gradually expanded and total expenditures have continued to increase (Table 2). With the growing number of patients and improvements in diagnostic capacity, however, potential operational pressures on the fund have also begun to emerge (Table 3). This indicates that while the system has effectively expanded risk pooling, it now faces new sustainability constraints, necessitating further optimization in areas such as dynamic adjustment of financing standards and drug price negotiation mechanisms.

3.3. Shifting from insurance-based coverage to multi-actor co-governance

In the problem stream, the National Healthcare Security Administration has continuously included rare disease medications in the NRDL, but a substantial number of patients still face a "coverage gap" due to the high costs of unlisted medications. With the ongoing emergence of new drugs and technologies, the financial burden on patients continues to rise. The existing special fund has limited capacity to absorb additional risks. There is thus an urgent need to explore new multi-tiered medical security mechanisms with broad societal participation.

In response, within the policy stream, the policy community began exploring multi-departmental approaches to medical security for rare diseases outside the scope of the NRDL. A mechanism of shared responsibility across society was developed, integrating diverse social resources. Specifically, out-of-pocket expenses for rare disease treatments not covered by the NRDL were covered by Zhoushan's multi-tiered subsidy, thereby creating additional space for local policy innovation within the existing national policy framework.

In the political stream, public expectations of "common prosperity" and improved social welfare provided normative support for the participation of social actors. At the same time, national and provincial policies promoting the development of philanthropy and voluntary donations to charity ("third distribution") offered institutional empowerment for local innovation. Acting as policy entrepreneurs, the Zhoushan Municipal Healthcare Security Administration and related departments seized the policy window by aligning the "multi-actor co-governance" scheme with the broader policy narrative of common prosperity, thereby facilitating rapid policy adoption.

In April 2025, the Zhoushan Municipal Healthcare Security Administration, together with the Zhoushan Civil Affairs Bureau and seven other departments, jointly issued the Notice on Establishing and Improving a (Trial) Multi-tiered Subsidy Mechanism for Rare Disease Medical Expenses, marking the successful convergence of the three streams (*24*). This mechanism establishes a tripartite linkage framework consisting of "social charity, departmental assistance, and special

Table 2. Zhejiang's special fund for rare diseases: Coverage by disease (units: 10,000 RMB, persons)

Disease	Drug	2020		2021		2022		2023		2024	
		Patients	Payment	Patients	Payment	Patients	Payment	Patients	Payment	Patients	Payment
Glycogen storage disease II	Myozyme	14	345.51	24	2,034.78	29	2,023.36	33	3,757.51	37	4,907.73
Fabry disease	Fabrazyme	6	197.44	17	954.29	29	1,118.85	46	2,392.17	56	3,168.79
Gaucher disease	Cerezyme	21	1,799.27	27	3,260.83	28	2,832.52	32	4,316.54	35	4,287.9
Phenylketonuria	Kuvan	15	32.01	23	59.92	18	22.22	–	–	–	–
Total		56	2,374.23	91	6,309.82	104	5,996.95	111	10,466.22	128	12,364.42

Table 3. Operational status of Zhejiang's special fund for rare diseases (units: 10,000 RMB)

Year	BMI Enrollment (units: 10,000 persons)	Fund Income	Patients Covered (units: persons)	Fund Expenditure	Fund Surplus	Cumulative Surplus
2020	5,054	10,108	56	2,374.23	7,733.77	7,733.77
2021	5,081	10,162	91	6,309.82	3,852.18	11,585.95
2022	5,577	11,154	104	5,996.95	5,157.05	16,743
2023	5,621	11,242	111	10,488.43	753.57	17,496.57
2024	5,713	11,858.4	128	12,364.42	-509.02	16,987.55

Abbreviation: BMI, Basic Medical Insurance.

fiscal support," with priority given to diseases that incur high out-of-pocket medical expenditures. Innovatively, it introduces a threshold of 50,000 RMB alongside a tiered sequence of resource interventions. Specifically, out-of-pocket expenses for rare disease treatments not on the NRDL are first partially reimbursed through "Zhouhuibao" (a form of commercial supplementary medical insurance). The portion exceeding 50,000 RMB is first covered through an initial round of assistance provided by temporary relief programs as well as by organizations such as the Federation of Trade Unions, the Disabled Persons' Federation, the Red Cross Society, and the Charity Federation in accordance with relevant policies. If the remaining individual burden after this first round still exceeds 50,000 RMB, a second round of support is provided by the special assistance fund for rare diseases, covering 30% of the excess, with an annual maximum subsidy of 100,000 RMB. Under this policy framework, commercial insurance providers progressively expand the range of rare disease drugs covered under "Zhouhuibao," thereby strengthening risk-sharing across multiple insurance schemes. In addition, this coordinated mechanism leverages the administrative hierarchy to break down data silos and it implements a "no-application-required" model of care. The medical security authority serves as a data hub, integrating medical expenditure information and regularly sharing it with relevant departments, enabling coordinated delivery of multi-tiered assistance and promoting a shift from a passive response to proactive governance.

Through this nine-department joint initiative, Zhoushan has expanded the scope of medical security from expenses covered by the NRDL to high-cost expenditures beyond the NRDL, effectively filling a critical institutional gap. The "no-application-required" model enhances patients' sense of accessibility while improving institutional sustainability. This transformation marks a shift in Zhejiang's medical security system for rare diseases from a government-led model toward a new stage of multi-actor collaborative governance.

4. Core policy arguments derived from the Zhejiang experience

4.1. Institutional layering is essential to medical security for rare diseases

Medical security for rare diseases cannot be effectively achieved through the mere expansion of BMI coverage. Rather, viable policy pathways typically entail the creation of a multi-tiered risk-sharing mechanism grounded in the logic of institutional layering (*25*). Zhejiang Province has progressively established a stratified medical security framework, with BMI as the foundational layer, critical illness insurance as an extension, and medical assistance as a safety net, complemented by special schemes for rare diseases and the participation of societal actors. Within this framework, BMI provides universal and routine coverage, critical illness insurance enhances reimbursement levels to mitigate catastrophic healthcare expenditures, and medical assistance provides a safety net for low-income populations. In addition, a special rare disease fund offers targeted financing for high-cost orphan drugs, while commercial supplementary insurance and charitable contributions further bridge the financing gap for medical expenses not covered by the NRDL. Through such a multi-layered institutional configuration, high-cost medical expenditures are effectively redistributed across different tiers of the system. This arrangement not only alleviates the systemic fiscal pressure on the BMI fund but also helps to enhance the stability and sustainability of medical security for rare diseases.

4.2. State-embedded policy entrepreneurship reshapes classic MSF assumptions

The Zhejiang case demonstrates that, under a highly centralized and administratively led governance structure, the operational logic of the MSF requires contextual adaptation. In the course of policy development, policy actors in Zhejiang strategically embedded the issue of medical security for rare diseases into the broader national policy narrative. Initially framed within the policy agenda of targeted alleviation of poverty and the prevention of illness-induced poverty, the issue was subsequently integrated into the overarching goals of "common prosperity" and the development of a multi-tiered medical security system. By aligning technical healthcare financing arrangements with national policy priorities, these actors facilitated the convergence of the problem, policy, and political streams, thereby creating critical conditions for the opening of policy windows.

This pattern—where core administrative agencies (*e.g.* the Healthcare Security Administration) function as "institutionally embedded policy entrepreneurs"—differs markedly from Western models that rely more heavily on external interest groups. It reduces the transaction costs of cross-sectoral coordination and highlights the capacity of administrative actors to proactively align political objectives with policy design (*26*). Such an approach constitutes a key driving force behind the effective implementation of complex healthcare policies.

4.3. Addressing "expenditures for drugs not on the NRDL" as a new frontier of policy innovation

Under the stringent state regulatory framework, local governments possess limited discretion over reimbursement for care covered "by the NRDL," hampering the provision of adequate security from high-cost medical expenditures. The practices in Zhoushan, Zhejiang Province, indicate that a critical entry point for policy innovation lies in dealing with high-cost expenditures for drugs not on the NRDL. By reconstructing the local governance network, this approach systematically integrates commercial supplementary medical insurance, temporary assistance from civil affairs, policy subsidies from organizations such as the Federation of Trade Unions, the Disabled Persons' Federation, the Red Cross Society, and the Charity Federation, and special subsidies for rare diseases. Together, these sectors collaboratively bear the exorbitant medical expenses not yet covered by the BMI. This model extends the focus of medical security from "drugs on the NRDL" to "drugs not on the NRDL," effectively bridging existing coverage gaps through a mechanism of multi-actor collaboration. It offers a replicable policy pathway for other regions seeking to manage medical expenditures outside the scope of the NRDL and to institutionally innovate approaches to medical security.

5. Policy implications for China and beyond

5.1. Designing sustainable rare disease funds under fiscal constraints

Against the backdrop of limited fiscal resources, establishing a provincial-level special fund for rare diseases is both feasible and stable, provided that it has an institutional financing mechanism. The experience of Zhejiang demonstrates that introducing a symbolic and minimal contribution within a broader pool of medical insurance funds—such as deducting a very small annual amount per capita (*e.g.* 2 RMB) from the critical illness insurance fund—enables the creation of a pooled fund of significant scale without imposing a noticeable burden on the public, thereby ensuring a stable source of financing for high-cost rare disease treatments. International experience likewise indicates that special funding mechanisms constitute an important policy instrument for addressing the high costs of rare disease treatment. Examples include Australia's Life Saving Drugs Program (LSDP), Japan's Nanbyo Medical Care Subsidy System, and Russia's High-Cost Nosologies Program. These systems all provide substantial medical security for patients with rare diseases through specific financial arrangements (*27,28*).

However, stable financing alone does not guarantee the long-term sustainability of such funds. With improvements in diagnostic capabilities and the continuous introduction of innovative therapies, related expenditures are likely to increase rapidly, placing persistent fiscal pressure on public healthcare systems (*29*). The sustainable operation of special funds therefore requires complementary institutional tools, such as systematic pharmaceutical price negotiations, health technology assessment (HTA), and dynamic management of reimbursement lists. Integrating clinical efficacy, cost-effectiveness, and payment decision-making enables the control of fiscal risks while prioritizing access to high-value therapies.

5.2. Balancing equity and efficiency for high-cost, low-prevalence conditions

One of the core challenges in rare disease policy lies in how to achieve a balance between equity and efficiency with limited public healthcare resources (*30*). Policymakers should carefully navigate the trade-off between "expanding the scope of disease coverage" and "providing patients with substantive financial protection."

In its institutional design, Zhejiang Province has introduced mechanisms such as caps on out-of-pocket expenditures and a tiered reimbursement structure with progressive rates (*31*), alongside multi-departmental, stratified subsidies for high-cost rare disease treatments that are not on the NRDL. These policy instruments offer valuable practical insights into addressing the equity–efficiency dilemma. By setting explicit ceilings on out-of-pocket payments and gradually increasing reimbursement ratios across expenditure brackets while simultaneously mobilizing multiple departments to provide targeted support for high-cost, out-of-pocket medical expenses, resources can be more effectively directed toward patients facing catastrophic health expenditures, thereby reflecting a clear principle of vertical equity. At the same time, to prevent declines in allocative efficiency, such payment policies typically require rigorous oversight by expert review committees or HTA bodies. These entities conduct comprehensive evaluations of the clinical efficacy and cost-effectiveness of drugs and treatment options prior to their inclusion within the support framework (*32*). Such mechanisms of institutional assessment not only safeguard patient interests but also help to maintain the operational

efficiency and fiscal sustainability of the medical security system.

5.3. Conditions for and limits of policy transferability across jurisdictions

The Zhejiang model offers significant empirical insights into the development of a national-level medical security framework for rare diseases. Moreover, the design of its diversified, multi-tiered security system echoes international policy principles regarding risk-sharing of medical expenses for rare diseases. Notably, the provincially pooled special fund and multi-departmental approach to medical security necessitate that local governments possess relatively robust fiscal capacity and substantial funding pools for BMI or critical illness insurance; otherwise, creating adequate risk-pooling capacity will be difficult. Additionally, the effective implementation of such policies hinges on a mature medical security system, highly institutionalized mechanisms of cross-sectoral collaboration, and the government's strong commitment to social equity. Despite these prerequisites and contextual limitations, Zhejiang Province's practical experience could still serve as an instructive policy blueprint for the creation of a robust medical security system for rare diseases.

6. Conclusion: Towards adaptive and multi-level management of rare disease care

Medical security for rare diseases should be conceptualized as a system of dynamically evolving policies. An analysis based on the MSF indicated that this system is continuously driven by the dual forces of advances in medical technology and shifting societal expectations, thereby requiring ongoing institutional adjustments in response. The evolution of medical security policies for rare diseases in Zhejiang Province exhibits distinct stage-based and adaptive characteristics. Institutional adjustments at each stage have not only responded to practical governance demands but have also laid a solid foundation for subsequent policies.

Within a highly centralized healthcare system, policymakers should maintain institutional flexibility and the capacity for policy innovation. When the NRDL cannot be rapidly expanded, policy innovation often needs to rely on supplementary institutional arrangements, such as the establishment of special funds or the exploration of mechanisms for multi-actor collaboration. The multi-departmental approach to medical security in Zhoushan, Zhejiang Province, has shifted the focus of coverage from reimbursing the cost of drugs on the NRDL to targeted compensation of expenses for drugs not on the NRDL. This reflects a form of strategic policy innovation in an existing institutional space and effectively bridges the gaps in the medical security system.

In the future, policy initiatives should focus on advancing higher-level risk pooling mechanisms and exploring the establishment of a national-level special fund for rare diseases in order to enhance medical security and promote regional equity. In addition, further steps should be taken to encourage the participation of societal actors, enabling supplementary mechanisms such as commercial insurance and charitable donations to produce a stable and coordinated paradigm within the medical security system for rare diseases (*33*). By continuously refining multi-actor collaboration and risk-sharing mechanisms, a more equitable and sustainable medical security system for rare diseases can be steadily created.

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**Address correspondence to:*
Pingyang Wang, Zhejiang Medical Security Research Association, 6th Floor, No. 100 Fengqi Road, Gongshu District, Hangzhou 310000, China.
E-mail: waterwm@126.com

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