Policy Forum

DOI: 10.5582/irdr.2025.01058

The imperative for national legislation on rare diseases in China: A policy review and call to action

Yangyang Li[1,*], Zheng Liu[2], Rufang Huang[1], Yugang Liu[2]

[1] Chinese Organization for Rare Disorders (CORD), Beijing, China;
[2] Simon-Kucher & Partners, Global Strategy and Marketing Consulting, Shanghai, China.

SUMMARY: Rare diseases represent a significant public health challenge in China, affecting an estimated 20 million individuals. Despite incremental policy improvements over the past decade, including the publication of two National Rare Disease Lists, an increasing number of available treatments, and the inclusion of some therapies in the Nationally Reimbursed Drug List (NRDL), patients continue to face systemic challenges in diagnosis, treatment access, and sustainable protection. That said, China has very limited rare disease research & development (R&D) and industrial development, so the market potential is far from being tapped. This policy review argues that the lack of a national legal definition for rare diseases and orphan drugs, an unsustainable payment mechanism for high-value innovative therapies, and insufficient incentives for domestic research and development have collectively hindered the creation of a sustainable rare disease ecosystem. Drawing on an analysis of patient registry data, policy documents, and proposals from China's National People's Congress (NPC) sessions, we demonstrate a growing societal consensus on the need for comprehensive national legislation on rare diseases, which is not only a moral imperative to safeguard the rights of patients but also a strategic necessity for a national population strategy and biomedical industrial development. We consider systemic rare disease legislation in China to be imperative, and now is the optimal time to promote rare disease legislation in China. We propose nine key initiatives, including establishment of a working committee on national legislation, creating a standardized definition of rare diseases and orphan drugs, creating a dedicated national rare disease fund, and robust R&D incentives.

Keywords: rare diseases, health policy, legislation, China, orphan drug, biomedical industry

1. Introduction

Rare diseases collectively impact approximately 20 million people in China, creating substantial challenges for patients, families, and the healthcare system within a rapidly changing healthcare landscape (*1*). The Chinese Government has acknowledged this challenge, enacting pivotal measures since 2018 such as the creation of the two installments of National Rare Disease Lists, which consist of 207 diseases to date (*2-4*), the establishment of a national diagnosis and treatment collaboration network, facilitation of the market entry for over 100 rare disease drugs, and around two-thirds of in-market rare disease drugs have been included in the Nationally Reimbursed Drug List (NRDL).

However, these well-intentioned efforts, often siloed within individual ministries or regional governments, lack the cohesion and authority of overarching legislation. This policy fragmentation has resulted in persistent gaps in diagnosis and care, treatment accessibility, social inclusion, and a stagnant domestic innovation landscape. This article reviews the limitations of the current policy framework, underscores the strategic opportunity the rare disease sector presents, and makes the case that comprehensive national legislation is the essential next step to transform the lives of millions and secure China's position as a leader in biomedical innovation.

2. The persistent challenges for patients

Despite significant advances over the past years, rare disease patients in China still confront persistent and multifaceted challenges to their survival and well-being.

2.1. Diagnostic odyssey

Of the over 7,000 known rare diseases globally, only 207 are listed in China's Rare Disease List (*2-4*). The path to a definitive diagnosis could be notoriously long and fraught with errors. A cross-sectional analysis

of the 2018 national rare disease survey in China, involving 1,010 adult patients with rare diseases, indicated that the average diagnostic odyssey was 4.30 years, with a misdiagnosis rate of 72.97% (*5*). This is compounded by a critical shortage of medical expertise; an article published in 2021 indicates that only 5.3% of physicians reported being "moderately or well aware" of rare diseases while the majority suspected rare diseases in patients fewer than 3 times (*6*).

2.2. Therapeutic desert – poor treatment availability

Although therapies for an increasing number of rare diseases have been approved internationally, fewer than 10% of the 7,000+ known rare diseases have specific treatments worldwide, and access in China remains limited (Table 1). For China's First Rare Disease List, which includes 121 conditions, 116 drugs approved by the U.S. Food and Drug Administration (FDA), European Medicines Agency (EMA), or China's National Medical Products Administration (NMPA) are available on the Chinese market, effectively addressing 53 distinct rare diseases (*7*). Among the 86 conditions on China's Second List of Rare Diseases, only 37 (43%) have drugs with approved indications domestically, and 10 diseases have therapies approved by the FDA or EMA that are used off-label in China (*8*). Together, these figures highlight the persistent challenges in providing timely and equitable access to rare disease treatments across the country.

2.3. Limited treatment accessibility – lack of a systematic payment solution for innovative therapies

China has established a national social insurance system, Basic Medical Insurance (BMI), which covers approximately 98% of the population. The NRDL plays a critical role in determining which therapies are eligible for reimbursement under BMI. However, treatment solutions with annual costs exceeding RMB ¥500,000 (USD $70,000) for negotiation or RMB ¥300,000 (USD $42,000) for reimbursement are typically excluded from NRDL coverage (*9*). Although the inclusion of rare disease therapies in the NRDL has increased in recent years, reflecting growing but still selective efforts to cover the reimbursement of these drugs, significant gaps in access remain (*10*). A recent analysis found that 34 approved rare disease drugs are not reimbursed by health insurance (*11*), including many high-cost, specialized treatments that

Table 1. 33 Rare diseases for which there are "drugs approved abroad but not yet approved in China"*

No.	Disease	FDA approval	EMA approval
1	Hereditary epidermolysis bullosa	√	√
2	Hypophosphatasia	√	√
3	Laron syndrome	√	√
4	Leber hereditary optic neuropathy	×	√
5	Lysosomal acid lipase deficiency	√	√
6	Porphyria	√	√
7	Very long-chain acyl-CoA dehydrogenase deficiency	√	×
8	Achondroplasia	√	√
9	Adult-onset Still's disease	√	√
10	Alpha-1 antitrypsin deficiency	√	×
11	Bardet-Biedl syndrome	√	√
12	Renal clear cell sarcoma	√	×
13	Cold agglutinin disease	√	√
14	Merkel cell carcinoma	√	√
15	Cystinosis	√	√
16	Eosinophilic gastroenteritis	√	√
17	Epithelioid sarcoma	√	×
18	Fibrodysplasia ossificans progressiva	√	×
19	Progeria	√	√
20	Leber congenital amaurosis	√	√
21	Limbal stem cell deficiency	×	√
22	Metachromatic leukodystrophy	√	√
23	Neuronal ceroid lipofuscinosis	√	√
24	Osteosarcoma	×	√
25	Pheochromocytoma	√	×
26	PIK3CA-related overgrowth spectrum	√	×
27	Primary insulin-like growth factor-1 deficiency	√	√
28	Recurrent pericarditis	√	×
29	Rett syndrome	√	×
30	Tenosynovial giant cell tumor / pigmented villonodular synovitis	√	×
31	Thrombotic thrombocytopenic purpura	√	√
32	Tumor necrosis factor receptor-associated periodic syndrome	√	×
33	Von Hippel-Lindau syndrome	√	×

*Data originally from *Reference 14*, and updated by the authors of this article.

may be the only effective options for certain conditions (Table 2). These gaps exacerbate the financial burden on patients, who often rely heavily on family support. A 2021 Chinese patient survey reported that only 4.7% of rare disease patients held full-time employment, while 43.8% were children or otherwise dependent, highlighting the ongoing accessibility challenges faced by patients even when therapies are approved and available on the Chinese market (*12*).

2.4. Socioeconomic marginalization and intergenerational impact

The consequences of inadequate care extend far beyond health. From 2014 to September 2024, 26,304 rare disease patients have registered with the Chinese Organization for Rare Disorders (CORD). An analysis of 5,810 patients in the registry in 2019 revealed that the lack of timely treatment led to high rates of disability; over half of registered patients reported varying degrees of physical disabilities, indicating that disability is a major burden among this population (*13,14*). This, combined with pervasive stigma and a lack of support systems, creates a vicious cycle of illness-induced poverty, illness-induced unemployment, and recurring poverty due to illness, resulting in significant unemployment among patients. Without effective intervention, nearly one-third of children with rare diseases die before the age of five, a tragic outcome for the affected. The economic impact could be devastating. Data from the CORD registry also revealed that over 80% of families have an annual income below USD 7,000 (RMB 50,000), yet annual treatment costs consume 80% or more of their household income (*13,14*). This far exceeds the World Health Organization's threshold for catastrophic health expenditure (40%) (*15*), forcing families to make the difficult choice between treatment and basic sustenance.

This triad of challenges — inaccessible diagnosis, unaffordable treatment, and debilitating socioeconomic marginalization — underscore the urgent need for a systematic solution through national legislation.

3. The unrealized market potential of the rare disease sector

Evaluate Pharma projects that orphan drugs will make up a fifth of the forecasted USD 1.6 trillion in worldwide prescription drug sales by 2030 (*16*). Over half of new drugs approved in the US and EU are now designated as orphan drugs, driving a significant portion of biomedical innovation. Many blockbuster drugs began with a rare disease indication before expanding into broader markets. China possesses unique advantages to capitalize on this trend, with the world's largest patient population for clinical research, a rapidly advancing biotech infrastructure, and sufficient resources to make a significant impact. Many rare diseases are ideal targets for cutting-edge modalities like gene therapy, offering China a chance to leapfrog as the leader for the next generation of medicines. Moreover, breakthroughs in rare diseases frequently provide insights and therapeutic platforms applicable to common diseases, helping to propel the entire biomedical sector. An analysis from Boston Consulting Group (BCG) projects that, drawing from

Table 2. 16 Rare diseases for which "drugs are available but not reimbursed" and the "cost of treatment"*

Disease	Estimated Prevalent Patient #	Treatment	Annual cost of treatment (RMB 10,000)	
			Adult	Pediatric
Pompe disease	35,000	Alglucosidase alfa for injection	175	98
Mucopolysaccharidosis I	18,900	Laronidase solution for injection	311	139
Mucopolysaccharidosis II	27,440	Idursulfase injection	333	111
Mucopolysaccharidosis IVa	4,667	Elosulfase alfa injection	234 (withdrawn)	110 (withdrawn)
X-linked hypophosphatemia	70,000	Burosumab injection	156	106
Primary hemophagocytic lymphohistiocytosis (HLH)	28,000	Emapalumab injection	256	106
Primary light chain Amyloidosis	14,000	Daratumumab injection (subcutaneous)	45.7	—
Neuroblastoma	14,000	Dinutuximab beta injection	—	—
Neuroblastoma	14,000	Naxitamab injection	—	—
Alagille syndrome	46,667	Odevixibat oral solution	—	—
Transthyretin amyloid polyneuropathy (ATTR-PN)	7,600	Tafamidis meglumine soft capsule	47	—
Neurotrophic keratitis	700,000	Cenegermin eye drops	14 (withdrawn)	—
Neonatal hypoxic respiratory failure with pulmonary hypertension	—	Inhaled nitric oxide	—	155
Argininemia	4,000	Glycerol phenylbutyrate oral solution	60	36
Citrullinemia type I	6,363	Glycerol phenylbutyrate oral solution	60	36
Ornithine transcarbamylase deficiency	25,000	Glycerol phenylbutyrate oral solution	60	36
HHH syndrome	4,000	Glycerol phenylbutyrate oral solution	60	36

*Data originally from *Reference 14 and 22*, and updated by the authors of this article. #Numerical values here are based on average weights per disease area.

China's proportion of the global oncology market (approximately 5% to 7%), China's rare disease drug market is expected to reach a scale of 60–90 billion RMB by 2030 (*17*).

However, the huge market potential has not yet been tapped in China. Lacking legislation and policy support, few researchers have incentives to conduct research and development in the area of rare diseases, and few companies are willing to develop or market rare disease drugs. According to an analysis by the CORD, only 42% of the marketed 252 rare disease drugs are domestic products, and more than 80% of these domestic products are generics or biosimilars.

4. Root cause: Lack of systematic policy support

The root cause behind the persistent challenges for patients and the untapped market potential of the rare disease sector is the same: lack of systematic policymaking. China's existing responses can be characterized as decentralized, department-specific initiatives that lack synergy and long-term stability.

4.1. Lack of rare disease definition & listing dilemma

Since 2018, China's National Health Commission (NHC) has released two installments of Rare Disease Lists, which is a landmark step toward defining rare diseases in the country. However, as efforts in the rare disease field continue to progress, we also see that the limitations of this list-based approach have become increasingly apparent. First, the two installments include only 207 conditions — accounting for just 3% of the approximately 7,000 rare diseases identified worldwide. Secondly, the Rare Disease List remains a reactive and clinically-oriented policy tool that focuses primarily on conditions that are "diagnosable and treatable" (*18*). This creates a disincentive for research and development of new therapies for many rare diseases. Last but not least, the updating cycle has been slow and unpredictable. The second installment was published 5 years after the first installment. The Rare Disease List's exclusion criteria leave thousands of rare conditions unrecognized and thus ineligible for supportive policies.

4.2. Unsustainable payment models

The absence of a patient-centric and dedicated funding mechanism has created huge barriers to not only patient access but also industry development. China's BMI is primarily budget control-driven, and it is ill-equipped to finance innovative or ultra-high-cost therapies. The price cap of NRDL (an annual cost of no more than 300,000 RMB) has excluded most life-saving rare disease drugs, leaving patients in a predicament of extremely poor access (*10*). Moreover, the multi-tier payment system is based on the BMI and NRDL, if a therapy is not covered by NRDL, it is impractical for it to be funded by critical illness insurance, medical assistance, or commercial insurance within the existing institutional framework.

4.3. Insufficient research & development (R&D) incentives

Critical policies like the definition of rare disease and rare disease drugs, market exclusivity periods, and enhanced data protection for orphan drugs, though proposed in drafts, have not been formally enacted in China (*19*). This regulatory uncertainty fails to provide the long-term predictability needed for companies to commit to high-risk, high-investment rare disease R&D (*20,21*).

4.4. Uncoordinated ministries & central-local dynamics

On one hand, different ministries operate with conflicting priorities, hampering the creation of synergy. The NHC emphasizes "diagnosable and treatable" with the Rare Disease List, the National Healthcare Security Administration (NHSA) operates under a constrained budget, wary of formulary expansion that would increase financial pressure, while the National Medical Products Administration (NMPA) encourages broader "rare disease" drug development far beyond the Rare Disease List. This misalignment could stand in the way of cohesive policy development. On the other hand, the division of responsibilities between the central and local governments in rare disease development remains vague (*22*). Provincial-level rare disease funds in Zhejiang and Jiangsu used to be "best practices" in China but have now been suspended due to a lack of central guidance and support.

5. A consensus for legislative action

After more than 10 years of exploratory efforts, China needs to shift from fragmented actions to systematic legislation for rare diseases. First, China has amassed decades of experience in the rare disease field, with various government departments and local authorities having developed relatively mature policy-making expertise. Second, rare diseases have become a high-profile topic for proposals at the annual National People's Congress (NPC) and Chinese People's Political Consultative Conference (CPPCC) sessions, and a preliminary consensus has emerged across society regarding the necessity and urgency of legislation. Last but not least, drawing from the experiences of other regions globally, China now possesses the socioeconomic foundation required for enacting rare disease legislation. Most countries enacted rare disease legislation when their per capita GDP approached or

exceeded $10,000 — a threshold China had already reached by 2022. Moreover, several countries with lower per capita incomes than China have also successfully enacted rare disease legislation, such as Colombia and the Philippines.

Analysis shows that the number of publicly announced motions and proposals calling for rare disease legislation grew more than tenfold from 2019 to 2024 (*23-31*). The proponents of these proposals have expanded from primarily clinical experts to biopharmaceutical industry leaders, policy researchers, economists, and representatives from various social sectors (Figure 1). The content of these proposals has evolved from general calls for awareness to actionable policy blueprints. This multi-sectoral consensus underscores that rare disease legislation is now widely recognized not as an isolated healthcare issue, but as a critical national priority intertwined with China's social stability, economic development, and strategic positioning in the global biopharmaceutical industry. The collective voice from the NPC and CPPCC provides a powerful mandate and a clear roadmap for policymakers to initiate the legislative process.

6. Conclusion and recommendations: A framework for national legislation

National legislation for rare diseases can help harmonize disparate policies, guarantee sustainable funding, and send an unambiguous signal for innovation to the global community. Based on our extensive analyses and benchmarking, we propose a legislative framework built upon nine pillars:

i) Systemic rare disease legislation in China is imperative: Systemic legislation is the foundation for safeguarding the various social rights of rare disease patients and serves as a driving force for promoting quality population growth in China. At the same time, using rare diseases as an entry point can stimulate innovation in R&D, production, and services related to the rare disease industry, thereby advancing the development of new quality productive forces in biomedicine and fostering societal progress.

ii) Now is the optimal time to promote rare disease legislation in China: Rare disease legislation began globally in the 1980s, with most regions enacting it after their per capita GDP exceeded $10,000. China's level of economic development and social system have matured, fully meeting the conditions for rare disease legislation. Against the backdrop of intensifying geopolitical tensions and widespread critical reflection on Western systems due to global political and economic instability, China should seize the opportunity to demonstrate its soft power and value orientation by enacting legislation for minority groups, tailored to its national conditions.

iii) Nominate a national rare disease legislation working committee: A high-level task force or committee should be established at the central level to coordinate and promote rare disease legislation, break down inter-departmental barriers, pool collective wisdom, and develop a timeline and roadmap for the legislative process.

iv) Clarify the legal definitions of rare diseases and orphan drugs: Defining rare diseases and orphan drugs is the cornerstone of legislative efforts. The current mechanism for updating the Rare Disease List can no longer meet industry development needs. The definitions of rare diseases and orphan drugs should be

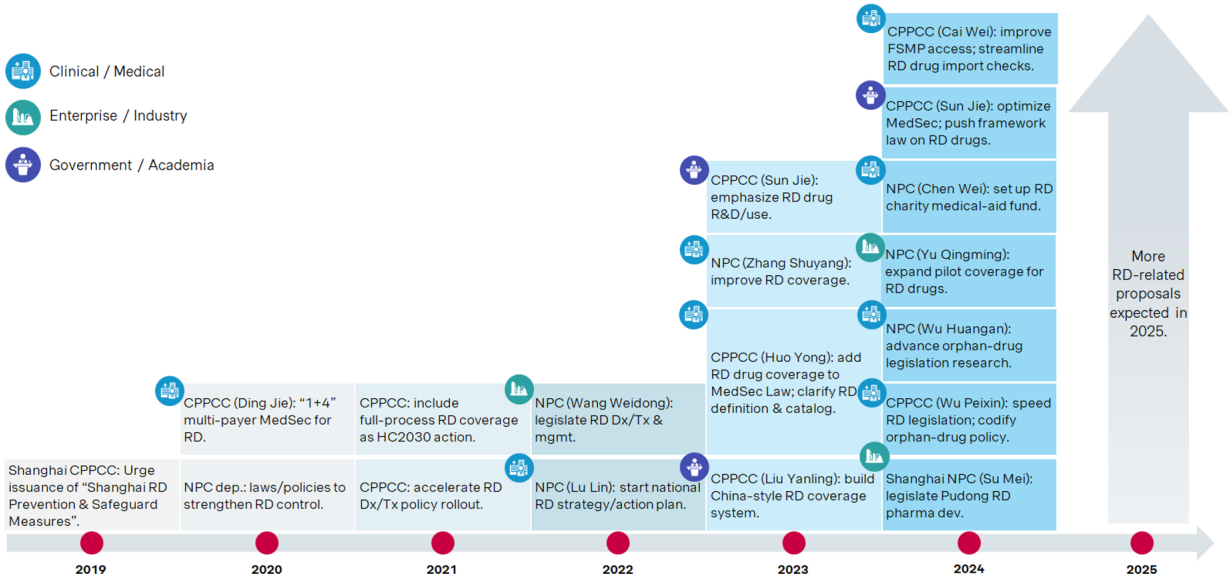

Figure 1. Trends in proposed legislation dealing with rare diseases in China. *Data Source: Ref. (23-31). Abbreviations*: RD, rare disease; Dx, diagnosis; Tx, treatment; R&D, research & development; NPC, National People's Congress; CPPCC, Chinese People's Political Consultative Conference; MedSec, medical security; HC2030, Healthy China 2030; FSMP, foods for special medical purposes.

swiftly discussed and confirmed under the leadership of the legislative task force, providing a legal basis for safeguarding the rights of rare disease patients and promoting industrial growth.

v) Establish a national special rare disease security fund: A national fund should be established specifically for rare diseases as soon as possible, integrating resources from various sectors (*e.g.*, the Ministry of Finance, Ministry of Civil Affairs, National Health Commission, and National Healthcare Security Administration) for fundraising and operation. In the meantime, local initiatives for special rare disease funds or other innovative payment models should be encouraged.

vi) Devise incentive policies for rare disease R&D and industrial development: Rare diseases sit at the pinnacle of medicine and addressing them involves tackling high-precision medical technologies and seizing the commanding heights of medical innovation. Rare disease legislation should explicitly outline incentives for R&D and industrial development, promoting the drafting and enactment of laws and regulations in key areas such as patent compensation, data protection laws, market exclusivity periods, and R&D tax benefits.

vii) Strengthen the national network for rare disease diagnosis, treatment, and rehabilitation: In recent years, China's national collaborative network for rare disease diagnosis and treatment has become an exemplary model globally. Legislation should further encourage the construction of a medical system for rare disease diagnosis, treatment, and rehabilitation, enhancing capabilities for early screening, diagnosis, and treatment. This will ensure rapid and accurate diagnosis of rare diseases and guarantee the accessibility and effectiveness of treatments.

viii) Fully safeguard the social rights of rare disease patients: Rare disease patients and families are deeply concerned not only with medical treatment but also with needs related to education, employment, and social integration. In formulating relevant regulations, the uniqueness of rare disease patients should be fully considered, ensuring their rights in healthcare, education, employment, movement, and social participation. These efforts will undoubtedly contribute to the further development of China's modern cities.

ix) Seize every opportunity to promote rare disease legislation at all levels: All government departments concerned with rare diseases should explore the formulation or revision of departmental regulations based on their functions. Regions with socio-economic conditions conducive to the drafting of legislation should examine their needs and capitalize on their resources to consider laws and regulations that protect the rights of rare disease patients and promote industrial development. When every department and region recognizes and responds to the needs of rare disease patients, taking actionable steps, these cumulative efforts will ultimately converge into a wave of progress for the era.

The moment to act is now. By enacting thoughtful and comprehensive rare disease legislation, China can secure the right to health for millions of its citizens, foster a world-leading biopharmaceutical industry, and demonstrate its commitment to building a truly equitable, innovative, and modern society.

Funding: None.

Conflict of Interest: The authors have no conflicts of interest to disclose.

References

1. Liu B. Embracing the change: Accessing rare disease patients in China. Simon-Kucher & Partners. *https://www.simon-kucher.com/en/insights/embracing-change-accessing-rare-disease-patients-china* (accessed September 1, 2025)
2. The Central People's Government of the People's Republic of China. Notice on publication of the first installment of the rare disease list. *https://www.gov.cn/zhengce/zhengceku/2018-12/31/content_5435167.htm* (accessed September 1, 2025) (in Chinese)
3. The Central People's Government of the People's Republic of China. Notice on publication of the second installment of the rare disease list. *https://www.gov.cn/zhengce/zhengceku/202309/content_6905273.htm* (accessed September 1, 2025) (in Chinese)
4. Tang M, Yang Y, Ye Z, Song P, Jin C, Kang Q, He J. Release and impact of China's "Second List of Rare Diseases". Intractable Rare Dis Res. 2023; 12:251-256.
5. Yan X, He S, Dong D. Determining how far an adult rare disease patient needs to travel for a definitive diagnosis: A cross-sectional examination of the 2018 national rare disease survey in China. Int J Environ Res Public Health. 2020; 17:1757.
6. Li X, Zhang X, Zhang S, Lu Z, Zhang J, Zhou J, Li B, Ou L. Rare disease awareness and perspectives of physicians in China: A questionnaire-based study. Orphanet J Rare Dis. 2021; 16:171.
7. Liu Q, Liu X, Wang S, Shang J, Tang Y, Zhang B. Research of accessibility of rare disease drugs based on the China's First List of Rare Diseases. Med J Peking Union Med Coll Hospital. 2023; 14:1208-1216. (in Chinese)
8. Liu Q, Liu X, Zuo W, Wang S, Zhang B, Zhang S. Study on drug list and accessibility of rare diseases based on the China′s Second List of Rare Diseases. J Rare Diseases.2024; 3:195-201. (in Chinese)
9. NRDL+. The future of innovative medicine pricing and reimbursement in China: Signals from the 2024 NRDL negotiations. *https://www.nrdlplus.com/the-future-of-innovative-medicine-pricing-and-reimbursement-in-china-signals-from-the-2024-nrdl-negotiations/* (accessed September 1, 2025)
10. Liu B. China NRDL 2023 readout: The seven-year itch. Simon-Kucher & Partners. *https://www.simon-kucher.com/en/insights/china-nrdl-2023-readout-seven-year-itch* (accessed September 1, 2025)
11. Luo C, Lv K, Peng X, Xuan J. Accessibility and

reimbursement coverage of rare disease drugs in China: Insights from the China's lists of rare diseases. Value Health. 2024; 27:S2.
12. Li X, Liu M, Lin J, Li B, Zhang X, Zhang S, Lu Z, Zhang J, Zhou J, Ou L. A questionnaire-based study to comprehensively assess the status quo of rare disease patients and care-givers in China. Orphanet J Rare Dis. 2021; 16:327.
13. Shao W, Li Y, Wang F, Zhu Y, Xiao L, Huang R. Current status and solutions for the accessibility of rare disease drugs in China. China Food Drug Admin Mag. 2019; 2:8-15. (in Chinese)
14. Chinese Organization for Rare Disorders & IQVIA. China rare disease drug accessibility report 2019.
15. Xin X, Guan X, Shi L. Research on rare disease security mechanism in China based on the affordability evaluation of 5 rare diseases. China Pharmacy. 2014; 25:404-407. (in Chinese)
16. Evaluate. 2025 Orphan drug report: Are orphans that different? *https://www.evaluate.com/thought-leadership/orphan-drugs-2025-report/* (accessed April 22, 2025)
17. Boston Consulting Group. China rare disease industry report. *https://mp.weixin.qq.com/s/3lhEl3rWIR4c3uhFU8Ughg* (accessed September 1, 2025). (in Chinese)
18. National Health Commission of the People's Republic of China. Notice on the procedures for drafting of the rare disease list. *https://www.nhc.gov.cn/yzygj/c100068/201806/8b5aff37992543eb919a4975c2bd1f6e.shtml* (accessed January 4, 2026). (in Chinese)
19. Wu T. Discussion of eight hot topics regarding the " Regulations Implementing the Drug Administration Law (Draft Revision for Solicitation of Comments)". Haiwen & Partners. *http://www.haiwen-law.com/35/591* (accessed April 22, 2025). (in Chinese)
20. Xie S, Liu Z, Li S. Current status and countermeasures of the legal system for rare diseases in China. J Rare Diseases Res. 2024; 3:405-410. (in Chinese)
21. Sun J, Bi J. Proposal calls for high priority on R&D and use of rare disease treatments to alleviate patient burden. The Economic Observer. *https://news.qq.com/rain/a/20230228A08DB100* (accessed April 22, 2025). (in Chinese)
22. Chinese Organization for Rare Disorders & IQVIA. Rare disease healthcare security in China: City report 2020 Edition.
23. Shi H, Zhou H, Wu Y. Voices of the Two Sessions, departmental responses and policy agenda setting — Based on 235 replies to the suggestions of NPC Deputies and Proposals of CPPCC Members on Rare Disease. *China Public Policy Review*. 2022; 2:70-83. (in Chinese)
24. China Alliance for Rare Diseases. "Rare diseases" became a hot topic at the Two Sessions. *https://www.chard.org.cn/detail/news/37* (accessed April 22, 2025). (in Chinese)
25. Beijing Evening News. Proceeding with the establishment of a healthcare security mechanism for rare diseases in China. *https://tech.sina.com.cn/roll/2020-05-21/doc-iircuyvi4269638.shtml* (accessed April 22, 2025). (in Chinese)
26. Ministry of Civil Affairs of the People's Republic of China. Multiple Members of the National People's Congress and the Chinese People's Political Consultative Conference: "Rare Diseases" Deserve Greater Visibility. *https://www.mca.gov.cn/zt/n353/n381/c1660845453525839874/content.html* (accessed January 4, 2026). (in Chinese).
27. Legal Daily. Improving the "no drugs available" dilemma and solving the security predicament: NPC deputies propose to accelerate legislation on rare diseases. *http://www.npc.gov.cn/npc/c2/c30834/202307/t20230725_430757.html* (accessed April 22, 2025). (in Chinese)
28. Rare Diseases in China. The National Two Sessions convene today, with 13 delegates already speaking out for rare diseases. *https://www.raredisease.cn/News/Info/14325* (accessed April 22, 2025). (in Chinese)
29. People's Political Consultative Conference News. Zhao Y, Guo S. At the "Two Sessions," delegates rally for the "rare." *https://www.rmzxw.com.cn/c/2024-03-12/3509023.shtml* (accessed April 22, 2025). (in Chinese)
30. Tencent News. At the 2024 National Two Sessions, multiple delegates and members heatedly discuss rare disease topics: Industry insights. *https://news.qq.com/rain/a/20240311A05RYP00* (accessed April 22, 2025). (in Chinese)
31. Rare Diseases in China. The National Two Sessions convene today, with 15 delegates already speaking out for rare diseases. *https://www.raredisease.cn/News/Info/23891* (accessed April 22, 2025). (in Chinese)

Received September 26, 2025; Revised January 6, 2026; Accepted January 15, 2026.

**Address correspondence to:*
Yangyang Li, Chinese Organization for Rare Disorders (CORD), 88 Liuxiang Road, Shiliu Center, Building 15, Room 103, Fengtai District, Beijing 100071, China.
E-mail: eileen.lli@outlook.com

Released online in J-STAGE as advance publication January 21, 2026.