

Protecting the socioeconomic rights and interests of patients with rare diseases based on an innovative payment mechanism

Amei He¹, Minxing Chen¹, Peipei Song², Qingyi Wu¹, Chunlin Jin¹, Jiangjiang He^{1,*}

¹ Shanghai Health Development Research Center, Shanghai Medical Information Center, Shanghai, China;

² National Center for Global Health and Medicine, Tokyo, Japan.

SUMMARY The pathogenesis of diseases in the field of rare diseases is complex. Many rare diseases have yet to be conquered worldwide, and there are still no effective treatments for most rare diseases, resulting in limited accessibility to medications. Over the past few years, China has been committed to ensuring the availability of drugs for rare diseases, both at the national level and in all sectors of society. Through National Healthcare Insurance Negotiations (hereinafter referred to as "national negotiations"), the National Healthcare Security Administration has included several drugs for rare diseases in medical insurance coverage, addressing some of the issues with medications for rare diseases. National Negotiations have reduced the prices of drugs for rare diseases to a certain extent, but they remain expensive for many patients. By the end of 2023, out of the 165 drugs for rare diseases that had been launched, 53 were still not covered by medical insurance, leaving patients with a significant financial burden. Addressing payment issues remains a current challenge, and various regions in China are actively exploring innovative methods of paying for rare disease care to protect the socioeconomic rights and interests of patients with rare diseases.

Keywords rare diseases, payment methods, national negotiations, special funds

1. Introduction

There is no universally accepted definition of rare diseases globally. In China, according to the 2021 Research Report on the Definition of Rare Diseases in China, diseases with an incidence of less than 1/10,000 in newborns, a prevalence of less than 1/10,000, and affecting fewer than 140,000 individuals are classified as rare diseases (1). Although rare diseases have a low incidence, China has a large population, and the number of people with rare diseases exceeds 20 million, so rare diseases are not rare in China. The majority of patients with rare diseases have no access to medications or cannot afford the cost of treatment, placing a significant financial and emotional burden on their families. Many households fall into poverty due to illness or return to poverty as a result of it. There are currently over 7,000 known rare diseases worldwide. According to statistics, there are no effective treatments for 95% of these rare diseases (2). By the end of 2023, based on the First List of Rare Diseases and the Second List of Rare Diseases, China had 165 drugs for rare diseases on the market, covering 92 types of rare diseases. However, the number of known rare diseases in China is approximately 1,400 (3).

China's efforts to protect patients with rare diseases began relatively late, with only some economically developed regions initially exploring protections for those patients. Since 2018, China has increasingly prioritized the protection of patients with rare diseases, introducing a series of policies to ensure patients have access to medications (4-14), as shown in Figure 1. In 2018 and 2023, China issued the First List of Rare Diseases (covering 121 diseases) and the Second List of Rare Diseases (covering 86 diseases), respectively (15-17), and these lists cover a total of 207 rare diseases. In 2020, the Opinions of the Central Committee of the Communist Party of China and the State Council on Further Reform of the Medical Insurance System proposed a multi-level medical insurance system. This system is centered around basic medical insurance, supported by medical assistance, and supplemented by additional medical insurance, commercial health insurance, charitable donations, and medical mutual aid (18). In 2024, the National Healthcare Security Administration proposed the establishment of a comprehensive "1+3+N" multi-level medical insurance system (19). The "1" refers to a basic information platform, which includes features such as "one person, one file" and "one drug, one code";

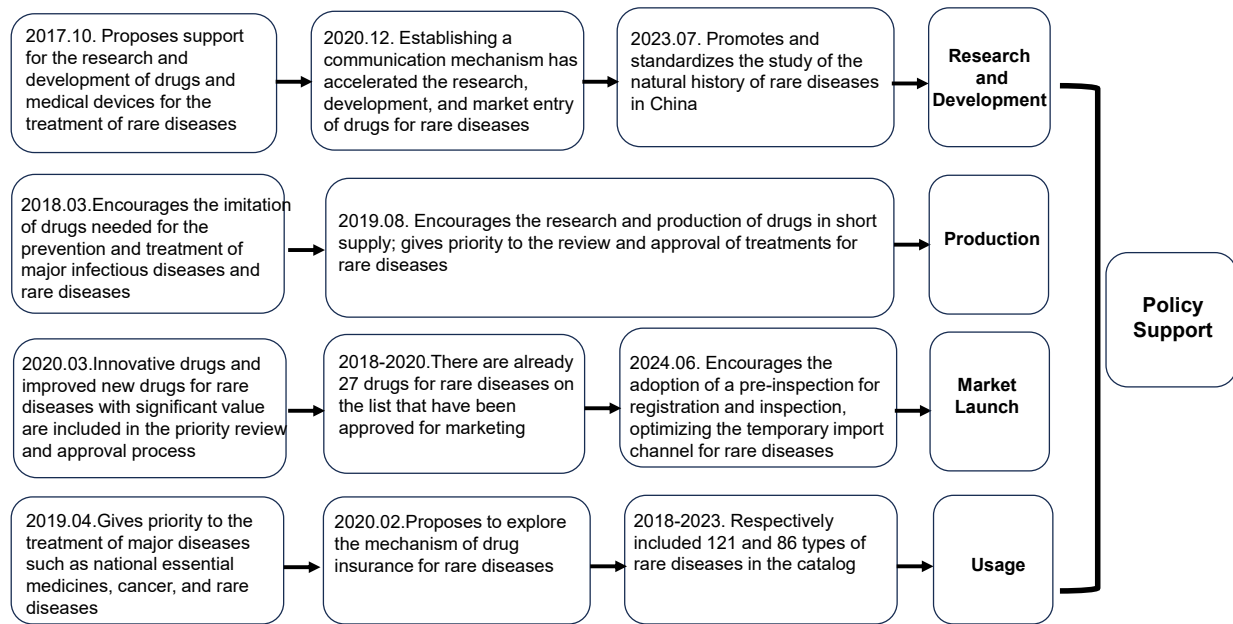


Figure 1. National policy documents on efforts to ensure the availability of drugs for rare diseases.

the "3" refers to the three levels within the basic medical insurance system, and the "N" represents commercial supplementary medical insurance, other forms of commercial health insurance, charities, funds, *etc.*

China has initially established a multi-level insurance system for rare diseases. However, the actual socioeconomic rights of patients with rare diseases are still in their infancy. From a health economics perspective, there are still many obstacles to overcome. Only by addressing issues related to funding and payment can the difficulties in diagnosis and treatment of patients with rare diseases be resolved, thereby protecting their socioeconomic rights.

2. Current status of the development of innovative payments for diagnosis and treatment of rare diseases in China

The small number of patients with rare diseases, the small market size, and the high research and development costs make drugs for rare diseases expensive. From an economic perspective, there is relatively insufficient motivation to research and develop drugs for rare diseases. Hospitals face pressure from assessments, and the extensive use of high-priced drugs may lead to rapid growth in indicators such as per capita costs, affecting their rating and thereby affecting the settlement of medical insurance funds. In order to address the issue of medications, various provinces and cities in China have explored multiple forms of payment methods. At the level of basic medical insurance, there are mainly one-way payment and outpatient management of special and chronic diseases. For outside medical insurance and

supplementary medical insurance, there are forms such as special funds, commercial supplementary medical insurance, medical assistance, and serious illness medical insurance, as shown in Table 1.

2.1. Accelerating the inclusion of and payments for drugs for rare diseases in medical insurance through national negotiations

As economic growth has enhanced social security, China has begun the work of including drugs in national negotiations (20). While better meeting the basic needs of insured individuals for medications for common diseases, drugs for rare diseases have been included in the scope of national negotiations since 2019, and several exorbitantly priced drugs for rare diseases have been successfully negotiated down in price. From 2019 to 2023, prices for a total of 42 drugs for rare diseases were successfully negotiated, as shown in Table 2. The 2023 version of the National essential drug list includes 112 drugs for rare diseases, covering 64 types of rare diseases. The treatment of rare diseases is mainly medication-based, and insurance for rare diseases has significantly improved.

In order to better negotiate drug prices nationally, China has introduced a "dual-channel" policy. The "dual-channel" refers to meeting the reasonable needs of ensuring the supply and facilitating the clinical use of negotiated drugs through two channels – designated medical facilities and designated retail pharmacies – and simultaneously incorporating them into the mechanism of medical insurance payment. Although the prices of medications for rare diseases have been reduced through

Table 1. Payment methods related to rare diseases

Name	Policy Content	Executing Area
Health insurance one-way payment	A policy established by the government, it is used to separately reimburse specific medical expenses. These expenses usually do not fall under regular reimbursement by hospital medical insurance funds.	Jiangsu Province
special and chronic disease outpatient management	This is established for certain diseases that have costly medical expenses, a clear diagnosis, can be treated on an outpatient basis, and that fall within the affordable scope of the medical insurance pooling fund.	Shanxi Province; Guangxi Province
Rare disease special fund	A fund dedicated to the financing and payment of the costs of rare disease treatment, designed to provide some sort of transitional protection	Zhejiang Province
Commercial supplementary medical insurance	This is commercial supplementary medical insurance, guided by local governments and jointly established by insurance companies and third-party operating platforms.	Shanghai's commercial supplementary commercial medical insurance
Other supplementary payment methods	Some regions have serious illness medical insurance or medical assistance systems for rare diseases. Medical assistance is a system in which the government gives special help and support to citizens who cannot obtain basic medical insurance or afford medical expenses due to poverty.	Shandong Province; Foshan, Guangdong Province

Table 2. National negotiations regarding medical insurance over the years

Year of negotiations	2015	2017	2018	2019	2020	2021	2022	2023
Number of qualifying entries	3	36	17	97	119	94	111	121
Average reduction	58.7%	44%	56.7%	60.7%	50.64%	61.71%	60.1%	61.7%
Number of drugs for rare diseases	0	0	0	7	6	7	7	15

Note: National Healthcare Insurance Negotiations of the People's Republic of China were organized by the National Health and Family Planning Commission in 2015, by the Ministry of Human Resources and Social Security in 2017, and by the National Healthcare Security Administration from 2018 to 2023.

national negotiations, fundamentally, these drugs remain expensive. In response to national negotiations over high-priced drugs, many provinces and cities have also implemented policies such as one-way payment and outpatient management of special and chronic diseases. For example, Jiangsu Province prioritizes the inclusion of drugs with long usage cycles, high treatment costs, and innovative drugs in "dual-channel" management and implements separate payment policies for some high-priced drugs without a deductible, while ensuring that the original benefits are not reduced (21). Shanxi Province has included some negotiated drugs in the provincial special drug catalog. For drugs for rare diseases within the catalog, including pulmonary arterial hypertension, multiple sclerosis, spinal muscular atrophy, Fabry disease, and acromegaly, the reimbursement rate has been increased by 10% compared to the original special drug policy, further ensuring the affordability for patients (22). The Guangxi Zhuang Autonomous Region has included 36 drugs for rare diseases suitable for outpatient treatment in the separate outpatient overall payment, benefiting patients with 27 types of rare diseases such as multiple sclerosis, narcolepsy, myasthenia gravis, neuromyelitis optica, and Gaucher's disease. An annual maximum payment limit is set, with a cap of 40,000 RMB for resident medical insurance and 80,000 RMB for employee medical insurance (23).

2.2. Payment for rare diseases from a special fund

Drugs for rare diseases are expensive, hampering their full coverage by China's basic medical insurance. Many experts have suggested establishing a dedicated fund for raising and paying for the costs of diagnosing and treating rare diseases (24). Currently, there is no special fund for drugs for rare diseases at the national level. Only some economically developed provinces and cities, such as Zhejiang, Jiangsu, and Qingdao in Shandong, have established special funds for rare diseases. By capping personal out-of-pocket expenses, these funds effectively address the accessibility of medication for patients. In Zhejiang, a sub-account is set up under the provincial medical insurance fund's special fiscal account. In accordance with the standard of 2 RMB per person per year, funds are transferred in one lump sum from the major illness insurance fund of the overall planning area to the Zhejiang Provincial Insurance Fund for Drugs for Rare Diseases. The maximum out-of-pocket expense for patients is capped at 100,000 RMB (25). Jiangsu Province has implemented provincial-level coordination and separate financing for insurance funds for drugs for rare diseases. It has established a multi-channel fundraising mechanism led by the government, with participation from market entities and charitable organizations. The insurance funds for drugs

for rare diseases are managed within the provincial fiscal social security special account, ensuring that they are used exclusively for their intended purpose and are independently accounted for (26).

2.3. Payment of commercial supplementary medical insurance

Through the 2024 Observational Report on Trends in the Rare Disease Sector in China, as of the end of 2023, more than 620 "commercial supplementary medical insurance" products have been launched in various cities across China, becoming an important part of the government-led creation of a "multi-level medical insurance system." Many insurance plans include coverage for drugs for rare diseases. For example, Shenzhen's commercial supplementary medical insurance further reimburses 80% of the drugs for rare diseases listed in the medical insurance catalog after deducting the deductible from the payments made by basic medical insurance. The annual coverage limit is up to 1.2 million RMB (27). Shanghai's commercial supplementary medical insurance further reimburses 70% after the basic medical insurance payment and deduction of the deductible, with an annual coverage limit of up to 1 million RMB (28).

2.4. Current status of additional supplementary payments

In some regions, medical assistance models for drugs for rare diseases have been explored. For instance, Foshan in Guangdong has implemented policies where, after reimbursement by basic medical insurance, major illness insurance, and various supplementary medical insurance plans, further assistance with the remaining personal payment for medical expenses is provided at a level of 80%, with an annual limit of 300,000 RMB (29). Shandong has implemented a major illness medical insurance coverage model for certain rare diseases, such as Gaucher's disease, Pompe disease, and Fabry disease, with segmented reimbursement, and the annual maximum reimbursement is up to 900,000 RMB (30).

3. Challenges and Perspectives

The affordability of medical insurance funds varies across China, and the high cost of medications for rare diseases inevitably impacts these funds. The epidemiological data for the diseases treated with these drugs, especially in terms of baseline information, is not sufficient. The estimated number of patients based on existing data or foreign indicators is not accurate, hampering the ability to precisely predict their impact on the medical insurance budget. Therefore, covering all medications for rare diseases under medical insurance is less feasible.

The primary factor in establishing a dedicated

fund for rare diseases is fundraising. However, with the numerous types of rare diseases and insufficient epidemiological data, calculating how much funding is required and finding sources for such funds has always been challenging. Setting up a dedicated fund requires the formulation of separate policies for rare diseases, which stokes controversy regarding fairness to patients with other diseases.

In conclusion, constructing a multi-party shared responsibility and diverse payment system has always been a key focus of China's. Regions are encouraged to calculate payments based on their own economic conditions and rare disease-related data and to innovate payment methods to protect patient rights, though this is a long and arduous task. As an important supplement to medical insurance, perfecting a diverse payment system is indispensable for commercial insurance. In the name of aiding patient in the information age, big data could be used to facilitate the exchange of disease data and reimbursement data by medical insurance departments of commercial insurance companies, enabling precise calculations. This, in turn, would allow for the development of appropriate payment mechanisms.

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*Address correspondence to:

Jiangjiang He, Shanghai Health Development Research Center, Shanghai Medical Information Center, No. 602 Jianguo (W) Road, Xuhui District, Shanghai 200031, China.
E-mail: hejiangjiang@shdrc.org

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