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Realistic Problems and Development Suggestions of China's Medication Assurance System for Rare Diseases

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Abstract

This study analyses the current situation and existing problems of China's rare disease medication Assurance practice in terms of policy system, main body's actions and protection effectiveness: from the perspective of policy design and the three main bodies' practical actions, the current situation of China's rare disease medication assurance is sorted out; based on this, the real problems of the current protection system in terms of the formulation of front-end policies and the actual benefits for end patients are clarified. Based on this, we will optimise the level of drug protection for rare diseases in China, and put forward specific recommendations: systematic construction of the top-level design for rare diseases, value assessment and payment agreement for innovative drugs in medical insurance, innovation and development of the medical emphasis system and talent cultivation, and improvement of the incentive system for market exclusivity and research and development of drugs for rare diseases.

Keywords

Rare diseases, medication for rare diseases, medication assurance.

1. Introduction

Rare diseases are characterised by complex etiology, multi-systemic morbidity, progressive development, high morbidity, mortality and disability, and most patients require lifelong treatment, which seriously affects their quality of life. Rare diseases are a medical and health problem, but also a social problem related to people's livelihood and well-being; drug therapy is the most important one among the limited treatment modalities for rare diseases. Therefore, for patients with rare diseases, access to appropriate medication is often the key to alleviating symptoms, delaying disease progression, or even changing the trajectory of their lives. Against the background of "difficult to diagnose, difficult to treat, and difficult to medicate", it is of great significance to put forward proposals to optimise the level of medication assurance for rare diseases based on the current situation and problems of medication assurance in China, in order to improve people's health.

2. Current Practice of Medication Assurance for Rare Diseases in China

2.1. Current Practice of Medication Assurance for Rare Diseases in China Section Headings

In recent years, the Chinese Government has devoted a great deal of attention to the protection of rare diseases, and various government departments have taken a number of measures to gradually overcome the difficulties encountered by patients with rare diseases in obtaining diagnosis, treatment and medication. The National Health Commission (NHC) has been improving diagnostic and treatment standards for rare diseases and raising the level of diagnosis and treatment of rare diseases nationwide by guiding the establishment of diagnostic

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and treatment co-operation networks and the implementation of information registries, among other things. The State Drug Administration (SDA) has issued a series of policies to accelerate the registration, review and approval process of drugs for rare diseases, and to support the research and development of drugs for rare diseases and the construction of the supply chain. The NHA has also adjusted its medical insurance catalogue to include more drugs for rare diseases in the reimbursement scope of medical insurance, and lowered the prices of some drugs through price negotiation strategies to reduce the financial burden of patients and improve the accessibility of drugs. In addition, the Ministry of Finance has issued tax incentives to encourage the production and importation of drugs for rare diseases, thereby further reducing the burden of drug costs on patients.

Meanwhile, since 2017, social attention to the issue of rare diseases has also increased significantly, and proposals related to the protection of rare diseases have been put forward in several consecutive sessions of the National People's Congress and the National People's Political Consultative Conference.

2.2. Establishment of a collaborative diagnostic and treatment network and promotion of information registration and disease screening

In terms of medical diagnosis, in recent years, the construction of the China Rare Disease Diagnostic and Treatment Collaborative Network and Information System has continued, narrowing the gap between China's rare disease diagnostic and treatment levels and those of European and American countries. The China Rare Disease Diagnosis and Treatment Network operates on a three-tier model of "national lead hospitals - provincial lead hospitals - member hospitals of the network", initially building a system of centralised diagnosis and treatment and two-way referrals, promoting the use of high-quality medical resources, playing the role of a radiation-driven organisation, and to a certain extent, improving the quality and efficiency of the services provided to patients with rare diseases.

Based on the diagnosis and treatment network, China has further required provincial health administrative departments and member hospitals of the collaborative network to carry out registration of rare disease cases, stipulating that the specific content of the registration includes the type of disease, the basis for diagnosis, the mode of registration, the time limit and the process, and other details. Through the collection of comprehensive and standardised data on rare disease cases, the registration of information can provide a scientific basis for understanding the epidemiology, clinical diagnosis and treatment of rare diseases in China, as well as for formulating intervention strategies and support policies for the population. In addition, China continues to improve its neonatal screening network for birth defects, including rare diseases. At present, the country has achieved full coverage of neonatal birth defects screening centres at the provincial level, which plays an important role in the prevention of rare diseases and other birth defects [1].

2.3. Explore multiple reimbursement models for medication payment and focus on patients' medication needs

At the national level, since the establishment of China's National Health Insurance Bureau (NHIB) in 2018, the health insurance catalogue has adopted a dynamic adjustment mechanism, usually once a year, which significantly shortens the time it takes for new medicines to be included in the health insurance catalogue, and responds in a timely manner to the people's need for medicines.The First Batch of the Rare Disease Catalogue and Second Batch of the Rare Disease Catalogue, which will be released in China between 2019 and 2023, cover 207 rare diseases . Among them, 112 medicines for 64 rare diseases have been included in the medical insurance, including 17 fully reimbursed Class A medicines and 95 Class B medicines reimbursed on a proportional basis; in the new version of the national medical insurance drug

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catalogue, which will be formally implemented from 1 January 2024, the number of medicines for rare diseases included in the catalogue has reached an all-time high, with a total of 15 medicines for rare diseases listed in the catalogue, which fills the gaps of 10 diseases in the medication coverage. A total of 15 medicines for rare diseases were included in the catalogue, filling the gaps in the medication coverage for 10 disease categories, and paying attention to the medication needs of patients with rare diseases to a certain extent.

At the local level, a multi-party co-payment protection policy for rare diseases has been initially introduced, focusing mainly on high-value innovative medicines for rare diseases that are not included in the catalogue. With years of exploration and development, the "special fund" model represented by Zhejiang, the "financial contribution" model represented by Qingdao, the "medical aid" model represented by Foshan, and the "medical assistance" model represented by Shenzhen have been gradually formed. The model of "special fund" represented by Zhejiang, "financial contribution" represented by Qingdao, "medical aid" represented by Foshan, "medical insurance for major diseases" represented by Shenzhen, and "sporadic supplementation of medical insurance" represented by Shanghai are typical models. While sharing some of the out-of-pocket pressure of rare disease patients, the practical exploration of the payment model for drugs outside the catalogue of rare diseases in various regions has also provided an experience reference for the State to improve the protection policy for rare diseases.

2.4. Carrying out basic research on drug innovation and accelerating drug development and marketing

To meet the huge market demand, in recent years, China has carried out a number of major scientific research projects related to rare diseases and set up special funds, focusing on rare diseases and other health problems and the development needs of the health industry, and has strengthened cutting-edge basic research, research and development of key technologies, transfer and transformation of achievements, development of medicinal products, and promotion of appropriate technologies, and the attention of pharmaceutical enterprises in the field of drugs for rare diseases has risen.

From an overall perspective, the coverage of diseases and the number of listed products in the field of rare disease drugs in China have increased significantly. According to the Drug Review Report 2023 published by CDE, 45 new rare disease drugs will be listed in China in 2023, of which 15 varieties (33.3%) will be accelerated through the priority review and approval process, and one will be approved for listing with conditions. At the same time, the situation that the rare disease drug market was dominated by multinational drug companies in the past has also been improved to some extent, and Chinese companies are gradually starting to make an impact. only 4 of the 27 rare disease drugs listed in the period of 2018-2022 were introduced or copied by domestic companies, while 18 of the 45 rare disease drugs newly listed in China in 2023 are already researched and developed and produced by local companies, covering 13 types of rare diseases. The Chinese rare disease market is expected to grow with the introduction or genericisation of domestic enterprises. It is foreseeable that as China's work in the field of rare diseases continues to advance, more Chinese enterprises will layout the field of rare diseases and further promote the listing of rare disease drugs.

3. Existing Problems with Rare Disease Drug Coverage in China

3.1. Fragmentation of government functions and conflicting safeguard policiese

Although the release of a series of policy documents has to some extent clarified the objectives and responsibility requirements for drug protection for rare diseases, China's current policy

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system for rare diseases still suffers from fragmentation and other problems. Based on the current management system, the three parties, the National Health Insurance Bureau (NHIB), the National Health Commission (NHC), and the State Drug Administration (SDA), are responsible for controlling the growth of medical costs, evaluating the performance of public hospitals, and overseeing and managing the supervision of medicines, respectively. Differences in the knowledge structure, functional scope and service targets of different functional departments can lead to the departments using their respective departmental performance appraisals as their administrative goal orientation, defining policy issues and taking corresponding actions from different value stances, perspectives of understanding, or applying different standards, with the key links not generating linkages, and with a disconnect between the three systems, and a failure to form synergies between the policy systems.

Take the issue of promoting access to rare disease medicines in the medical insurance catalogue as an example. On the one hand, the current "policy fight" between China's negotiated medicines and the assessment of medicines used by medical institutions affects the entry of negotiated medicines into hospitals. At present, the negotiated drugs are not counted in the ratio of drugs, the total budget of the fund, the medical income increase and other indicators of the scope of the assessment, the deletion of rare disease specification limitations and other requirements in the implementation of the process of lagging behind, and some of the assessment indicators and calculation methods within the medical institutions have not been synchronised to update [2]. On the other hand. Insufficient financial subsidies for public hospitals and the implementation of the zero-plus-fee policy for medicines have led to low initiative in equipping some medical institutions to use negotiated medicines. From the perspective of financial management, rare disease medicines are mostly high-value innovative medicines, most of which require special transport, storage and distribution measures. Public hospitals, which are "self-financing", often lack the motivation to use negotiated medicines that do not add to their revenues, but rather increase their costs, due to the difficulty of resisting profit-seeking motives. Against this background, the establishment of a synergistic system that integrates the resources and capabilities of the three health care providers is particularly important.

3.2. Insufficient number and low availability of rare disease medicines in the countrye

Based on patients' immediate interests, the availability of drugs for patients from the supply side is a prerequisite for drug therapy. China has made some progress in enriching the variety of drugs for rare diseases and improving the availability of drugs for patients with rare diseases by accelerating the listing of drugs for rare diseases [3]. However, the domestic rare disease drug research and development foundation is weak, the new drug research and development process is slow, and the problem of relying on expensive imported drugs is still serious [4], and there are problems such as "no drugs available", "drugs outside the country are available, but not approved within the country", "drugs approved within the country, but difficult to obtain", and "drugs approved within the country, but difficult to obtain". There are difficulties such as "no drugs available", "drugs available outside the country but not approved within the country", "drugs approved within the country but difficult to obtain". Data show that the listed rare disease medicines in mainland China only account for 58.9% of the listed medicines in the world, and 16 diseases with medicines outside China are facing the problem of not being listed in China; 24.2% of the listed medicines in mainland China have not yet been registered for rare disease indications, and patients with 20 types of rare diseases are still facing the problem that all medicines are for over-indications [5], and the number and types of medicines in China are far inferior to those in developed countries such as Europe, America, Japan, Australia and so on. Europe, America, Japan, Australia and other developed countries and regions [6]. The

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establishment and improvement of the entire rare disease drug research and development guarantee system, so that more patients with rare diseases can have access to drugs, still requires the joint efforts of the government, society, individuals and other parties.

3.3. The payment system for rare disease drugs is not yet sound, and the burden on patients is heavy

Due to the general attributes of "high cost, high risk and long cycle" of innovative drugs and the special attributes of higher market risk of rare drugs due to the small number of patients and the difficulty of diagnosis, manufacturers of drugs for rare diseases often charge high prices in order to compensate for the costs. Based on this, the most important thing is to ensure that patients can benefit from medicines that are affordable to them from the payment side. However, China does not yet have a sound medical insurance system for rare diseases, and some high-value rare disease medicines are not included in medical insurance or are not adequately compensated, which results in the high financial burden of medicines even if the medicines are available in China, affecting the accessibility of medicines to patients.

Specifically, on the one hand, there are still a variety of expensive rare disease medicines that are not covered by medical insurance in China. Statistics show that there are 22 diseases for which all therapeutic drugs are not included in the health insurance catalogue, including nine drugs that cost more than 500,000 RMB per year to treat. The number of patient registrations for these drugs is low, market expansion is difficult, and patients face a particularly high drug shortage and financial burden [7]. At the same time, patients in some regions that first explored the multiple coverage model for off-catalogue rare disease medicines also still face a heavy financial burden, with more than half of rare disease patients saying their families cannot afford the high medical costs [8], and there are problems in the pilot regions such as the system design not being comprehensive, and the effect of the policy not being significant [9]. On the other hand, even for rare disease medicines that have been included in medical insurance, the basic medical insurance is limited in the depth of compensation due to the solidified financing channels of the fund, and China's current medical insurance system has made less improvement in the affordability of rare disease medicines [10], and the payment system for rare disease medicines needs to be improved urgently.

In addition, to address the social problem of unaffordability of medication for rare diseases, China has failed to improve public service capacity through the partnership between the private and public sectors, and the government has failed to change its role from overall controller to participant and supervisor. For example, if we look at the payment and compensation subsystem, which is particularly important in the protection of medication for rare diseases, China still relies mainly on the basic health insurance to fulfil its function of "guaranteeing the foundation", and the role of commercial health insurance and charitable assistance in the multi-level system as a participatory force in the market and society has not been adequately fulfilled, resulting in a limited level of medical protection for patients with rare diseases.

3.4. Summary

To sum up, in recent years, China's rare disease medication assurance cause has stepped into the fast lane of development in recent years, and health insurance, medical care, and pharmaceuticals, as the main participants of rare disease protection, have actively laid out the problems of "difficult to diagnose, difficult to treat, and high cost" of rare diseases, and have made a lot of breakthrough results. However, there are still a series of problems in the development of rare diseases in China, such as fragmentation of policies, insufficient participation of stakeholders, and barriers to affordability and accessibility of medicines, and the level of medication assurance for patients with rare diseases still needs to be improved.

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In this context, considering the new era requirement of "three doctors' coordination" of "people's health as the centre" in the "Outline of Healthy China 2030" and the report of the 20th Party Congress, it is necessary to promote rare diseases through three-doctor coordination. It is necessary to promote the protection of medication for rare diseases through the synergy of the three medical institutions. Based on the guideline of "three-medicine synergy", it is possible for health insurance, medical care and pharmaceutical sub-systems to break through the complexity of interest entanglements and reach a common willingness to take action based on the common goal of safeguarding the health of people suffering from rare diseases; on the other hand, by allowing, encouraging and promoting the active participation of various social and market entities, cross-sectoral co-operation can make up for the limitations of the traditional drug protection system of rare diseases, which relies on a single government, and improve the effectiveness of drug protection system of rare diseases. government, and improve the level of drug protection for rare diseases. This is not only an effective response to current challenges, but also a positive layout for future development.

4. Suggestions for the Development of China's Rare Disease Medication Assurance System

4.1. Systematic construction of top-level design for rare diseases

It is recommended that a comprehensive law on rare diseases and rare disease medicines be introduced to regulate the definition of rare diseases, support for the research and development of medicines and therapies, protection of patients' rights and interests, and international cooperation, so as to enhance the emotional and value recognition of rare disease patients by the people and stakeholders. China should provide a clear definition of rare diseases based on the management of the rare disease drug catalogue. Referring to the developed countries and regions where the rare disease policy started earlier, they all regard the establishment of a definition of rare diseases in line with their own national conditions as the first measure to solve the problem of rare disease protection. By defining the meaning of rare diseases, it can provide a clear development direction and path for the pharmaceutical industry and related parties, and the qualification of drugs for rare diseases as the first step to promote the availability and affordability of drugs for rare diseases.

It is recommended that a specialised rare disease authority be set up to coordinate, supervise and manage activities related to rare diseases. Specifically, the functions of this organisation should include at least two aspects. First, policy implementation and supervision: responsible for drafting national policies on rare diseases, including R&D incentives, patient support programmes and international cooperation programmes; ensuring that policies are effectively implemented, and regularly evaluating the effectiveness of the policies and adjusting the direction of the policies based on feedback. Second, resource allocation and management: managing national funds allocated for rare disease research and treatment, ensuring the effective and fair use of funds; evaluating and approving rare disease research and drug development projects, ensuring that the projects are in line with national strategic goals and patient needs.

4.2. Innovative Medicare Assessment and Payment Agreements for the Value of Medicines for Rare Diseases

In order to fully reflect the value of drug innovation and clinical needs, innovative drugs for rare diseases in urgent clinical need should break through the standard method of health technology assessment of common drugs, and establish a comprehensive value assessment system with the characteristics of drugs for rare diseases and covering multiple disciplines such as pharmacoeconomics, medical science, security science, sociology, etc., so as to provide a

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reference basis for the access to the health insurance of China's rare disease orphans, and to incorporate more emergency-saving and lifesaving rare disease drugs into the basic health insurance catalogue, and prioritise and approve the projects for drug development. More drugs for rare diseases will be included in the basic medical insurance catalogue, and priority will be given to those rare diseases for which there are no alternative treatment options in the medical insurance catalogue. Specifically, the assessment system should be set up in such a way as to take into account such dimensions as clinical benefits, improvement in patients' quality of life, and long-term health cost savings; introduce a patient participation mechanism to ensure that the assessment system fully takes into account the actual needs of patients, while improving the comprehensiveness and transparency of the assessment; and design a dynamic data-tracking system to collect data on the actual use of medicines after they are included in the catalogue, including their efficacy, side-effects, and patient satisfaction. Use these data to regularly update the assessment results of medicines.

Based on the solidification of the financing channels of the basic medical insurance fund and the insufficient cost-effectiveness or evidence-based evidence of some rare disease drugs, which have led to a small number of rare disease drugs in the catalogue and a low level of payment and compensation for rare disease drugs, it is recommended that the national medical insurance department explore the use of innovative payment agreements, such as risk-sharing agreements based on the fund's risk or the risk of therapeutic efficacy, so as to incorporate more drugs for rare diseases into the basic medical insurance system in China. It is recommended that the national medical insurance department explore the use of innovative payment agreements based on fund risk or efficacy risk-sharing agreements, so as to incorporate more rare disease drugs into China's basic medical insurance system. Reference can be made to South Korea's practical experience in rare disease medication assurance and its risk-sharing agreement (RSA). In the agreement, the government and pharmaceutical companies share the risk of uncertainty about the clinical effects and budgetary impacts of new drugs after launch. In terms of the clinical effectiveness risk, the health insurance authorities can set specific terms with pharmaceutical companies on the therapeutic effectiveness of the drug, and if the drug's effectiveness is not up to standard, the pharmaceutical companies are required to offer discounts, price reductions or refunds to the payer. In this regard, the leading role of the government is key to realising multi-party co-payment, while capping patient out-of-pocket payments is an effective way to enhance patient benefits. In terms of financial risk to the fund, strategies such as capping the amount of drug reimbursement and the number of patient reimbursements can be implemented, thus directly controlling the burden of rare disease drug costs on the health insurance fund and assisting the health insurance department in more efficient budget planning [11].

4.3. Emphasis on rare disease healthcare system innovation and talent cultivation

In order to solve the problems faced by patients with rare diseases, such as difficulties in diagnosis, medical treatment and rehabilitation, and to achieve fair and accessible medical services, it is recommended to integrate the resources of China's current three-tier medical and healthcare service system to build a comprehensive service system of screening, diagnosis and treatment, rehabilitation, management, medical care, protection and social support for rare diseases, and to enhance the hard power of the medical system in the diagnosis and treatment of rare diseases. Specifically, it is recommended to strengthen the existing collaboration network for rare disease diagnosis and treatment, ensure that high-quality hospitals play an exemplary and leading role, and optimise the diagnosis and treatment process by sharing and interconnecting medical information through green referral, multidisciplinary consultation and other modes with the promotion and use of electronic medical records, telemedicine and other

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technologies. In terms of collaborative action, the establishment of the system requires close cooperation with medical institutions specialising in rare disease services, community organisations and social security workers, in order to jointly enhance the diagnosis and treatment capacity of grassroots and the level of social security.

In order to address China's current problems of insufficient awareness of rare diseases among grassroots clinicians and lagging behind in the construction of relevant departments, there is an urgent need for a systematic national programme to cultivate talents in the field of rare diseases and to encourage them to serve at the grassroots level. The goal of this programme should be to enhance the diagnostic, therapeutic and management capacity of medical personnel for rare diseases, especially in primary healthcare institutions, in order to improve the soft power to deal with rare disease protection, which is an important health and social issue. On the one hand, it is suggested to improve the rare disease talent education system. Courses related to rare diseases can be incorporated into the education system of medical colleges and universities, so as to ensure that future medical personnel have the necessary knowledge of rare diseases and the initial ability to deal with them before entering the clinical frontline treatment. On the other hand, it is recommended that the construction of national medical centres for rare diseases be accelerated and internship and training bases be set up in these centres, so that doctors can deepen their understanding of and ability to deal with rare diseases through practical learning. Through the establishment and operation of these centres, they can not only optimize the practical path of clinical diagnosis and treatment of rare diseases, but also serve as a distribution center for the latest national research results on rare diseases, promoting the quality improvement and equitable development of the entire rare disease protection system.

4.4. Improvement of rare disease drug market exclusivity and R&D incentive system

It is recommended to play a good combination of patent and data protection to extend the market exclusivity of rare disease drugs. In terms of patent protection, it is necessary to accelerate the implementation of the requirements for rare disease drugs and other drugs in R&D and production in the new Patent Law to be implemented in 2021, so as to balance the relationship between innovation and development of rare disease drugs, and to safeguard the research and development of rare disease drugs with a comprehensive patent protection network. In terms of data protection, reference can be made to the practice of Japan and the European Union to extend the time of data protection for rare disease drugs from six years to ten years for drugs targeting new chemical compositions in general; meanwhile, relevant supporting regulations should be issued as soon as possible for the release of the "Measures for Implementation of Data Protection for Pharmaceutical Trials (Provisional) (Draft for Public Opinion)" in 2018. Putting the data protection measures into practice is not only necessary for fulfilling the commitments made when China joined the World Trade Organisation, but also for encouraging biomedical innovation.

In addition, it is recommended that favourable policies, such as direct tax breaks, should be used to increase the incentives for enterprises to research and develop therapeutic drugs for rare diseases. The scope of rare disease varieties covered by tax incentives can be further expanded and the frequency of updating the list of incentives can be increased; new forms of exempting enterprises' income from sales of rare disease medicines and reducing or waiving the fees for drug registration applications can be explored to further incentivise enterprises to invest in R&D of rare disease medicines.

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