

China Rare Disease Drug Accessibility Report 2019

EDITORS IN CHIEF: KEVIN HUANG¹, ERIC SHAO²;
RESEARCHERS: EILEEN LI², GRACE WANG², MIRROR ZHU², LEI XIAO¹
ABSTRACT: MAKBULE TOP¹, QI SUN¹
¹CHINESE ORGANIZATION FOR RARE DISORDERS(CORD), ²IQVIA CHINA

INTRODUCTION

This report has been initiated by CORD, the Chinese Organization for Rare Disorders and IQVIA China. The collaboration began in November 2018 and was published as a report in 2019. The researchers interviewed total of 44 patients, Health care providers, rare disease researchers and chinese government officials and reviewed a wide range of secondary literature and conducted a thorough data analysis.

The overarching intention of this report is the attempt to „open up the path to the access of orphan medicines“ by firstly analysing the orphan drugs accessibility challenges that China’s rare disease patients are currently facing and secondly, as a result of the situation, providing a Strategy of Enhancing the Accessibility of orphan medicines in China by Formulating a National Action Plan.

Present abstract will give a summary of the current situation by providing the keynotes of the data output that this report has produced and secondly present the content of the National Action Plan as it is being suggested by CORD and IQVIA.

1. CURRENT CHALLENGES IN THE RARE DISEASES FIELD IN CHINA

1.1 NO LEGAL DEFINITION OF THE TERM „RARE DISEASES“

Currently there is no clear legal interpretation of the term „rare disease“. On May 12, 2010, the Medical Genetics Branch of the Chinese Medical Association held an expert seminar on the definition of China’s rare diseases in Shanghai. The experts proposed that rare diseases should be defined as diseases with an incidence of less than 1/500,000 or an incidence of less than 1/10,000 in newborns. Based on this definition, there are 16.8 million rare disease patients living in China.

1.2 LACK OF REIMBURSEMENT PAYMENT AS DILEMMA OF „DRUGS BEING UNAVAILABLE“

Rare disease drugs appeared in the past two round NRDL (National Reimbursement Drug List) national negotiations in China. Out of the 55 drugs, that are now available in China and have rare disease indications, 29 have been included in the NRDL involving 18 rare diseases. Among the 29 drugs, 9 are in the A List. Without the payment under medical insurance, patients will find it difficult to maintain sufficient treatment.

1.3 THE LAST MILE OF DRUG ACCESSIBILITY

Even if a drug is included in the NRDL, it does not yet mean that patients can gain smooth access to this treatment drug. Although orphan medicines are within reach, „the last mile“ to accessibility is still challenging. Apart from inclusion into the NRDL, great uncertainty still exists on a series of business access points for orphan drugs, such as provincial drug tendering, public hospital listing, restrictions on physicians’ prescriptions, outpatient reimbursement limitations, etc.

1.4 THERE IS AN URGENT NEED FOR THE ESTABLISHMENT OF A NATIONAL MEDICAL SECURITY SYSTEM

In 2005 Shanghai, Qingdao and Zhejiang started to explore the medical security models for orphan drugs and introduced the modes of ensuring orphan medicines with local characteristics, which helped alleviate the payment problem of patients to an extent. Following table summarizes the keypoints of these Major Local Pilots for Rare Diseases Healthcare Security in China:



QINGDAO: LED BY FINANCIAL PAYMENT AND SUPPLEMENTED BY MULTIPARTITE PAYMENT

MAJOR POLICIES
In 2005, rare diseases were included into the Critical Illness Insurance (CII) for outpatient service of urban employees.
In 2012, the critical illness aid system was implemented, relying on both financial contributors and medical insurance.
In 2013, a multi-level medical security system of „Basic Medical Insurance“ (BMI) + CII + supplemental medical insurance (SMI) was set up.

MODE OF FUNDRAISING

Qingdao set up the Special Fund for Medical Aid to Critical Illness by using its financial contributions shared by the financial department at the two levels of city and districts on a 1:1 basis. Without the need for insurance participants to make payment into the fund, the financial department will contribute 300 million yuan each year. In the amount, 200 million yuan is managed by the Healthcare Security Department for use in the aid to severely ill, specially medical devices and the high amount medical costs outside the coverage of medical insurance. 30 million yuan is left in the charge of the civil affairs department for use in the special aid to the patients in the families of low or middle income.

SHANGHAI: MULTIPARTITE ASSISTANCE THROUGH CHARITY FUNDS

MAJOR POLICIES
In 2011, the Children Hospitalization Mutual Aid Fund set up by Shanghai Red Cross, Education Committee and Health Family Planning Committee became the first to introduce the support policy for child patients of rare diseases.

In 2013, Shanghai Municipal Foundation for Prevention Treatment of Rare Diseases set up Shanghai Municipal Fund for Special Aid to Leprosy and Storage Disease.

In August 2018, the „Notice of Shanghai Municipality for Prevention, Treatment and Support of Rare Diseases (Draft)“, the first normative document of China for rare diseases, was put on the agenda.

MODE OF FUNDRAISING

Shanghai Hospitalization Mutual Aid Fund Payment is made for babies, infants and students of primary and secondary schools on a voluntary basis at the rate of no more than 100 yuan per year. Social Relief Fund (Special Storage Disease Management) donations.

ZHEJIANG: LED BY HEALTHCARE SECURITY DEPARTMENT WITH FINANCIAL FUNDS ENSURING BOTTOM LINE SUPPORT

MAJOR POLICIES
In 2015, the Department of Human Resources Social Security led the negotiations about the specialty drugs for rare diseases, incorporating 3 drugs into the reimbursement of DRG.

In 2017, contracts were renewed for 2015 products and additional two orphan medicines were included to DRG through negotiation.

MODE OF FUNDRAISING

The proportion of contribution is about 3 : 5 : 2 for departments of medical insurance, civil affairs and finance. The funds necessary for DRG are transferred in a lump at the beginning of a year out of the employee and urban – rural (UR) fund temporarily at the rate of 60% for the government or the employer and 40% for individuals.

4. APPENDIX: ESTIMATES OF CHINESE PATIENTS AND TREATMENT DRUGS AVAILABILITY OF THE DISEASES IN THE 121 CATALOGUE

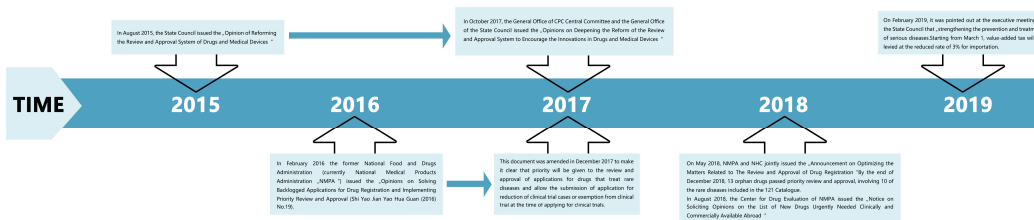
2. GOVERNMENT POLICIES THAT TRY TO OVERCOME SOME OF THE CHALLENGES

2.1 THE ISSUANCE OF THE „CATALOGUE OF FIRST BATCH OF RARE DISEASES“

May 2018 was an important day for the rare disease community because it was the first time that the Chinese government has defined rare diseases in the form of a catalogue of diseases. Five national departments, including The National Health Commission (NHC), the Ministry of Science & Technology, the Ministry of Industry and Information Technology, National Medical Products Administration (NMPA) and The State Administration of Traditional Chinese Medicine jointly issued the Catalogue of the First Batch of Rare Diseases („121 Catalogue“). According to the calculation based on epidemiological literature reports and further public data, the 121 rare diseases affect about 3 million patients in mainland China. Despite of this positive development, there are still many challenges that patients are facing, including the fact, that diseases are still hard to be diagnosed; there are no medical products available to treat the diseases after diagnosis; the treatment drugs have yet to be marketed in China or have no rare disease indications; the treatment drugs are expensive and have yet to be covered by medical insurance, though they are marketed or are hard to be included in prescriptions. Very few of the orphan medicines made by the world’s leading manufacturers are commercially available in China and market uncertainty is a very fundamental obstacle.

2.2 GOVERNMENT POLICIES AND MEASURES INTRODUCED SINCE 2015

A number of policies have been introduced since 2015 in order to counter market uncertainties. The lack of definition of rare disease drugs and an independent passage for review and approval of rare disease drug registration remains an invisible wall blocking the accessibility of orphan medicines. Additionally, the import, registration and launch of orphan medicines in China need the support of further supporting policies and regulations.



3. STRATEGY OF ENHANCING THE ACCESSIBILITY OF ORPHAN MEDICINES IN CHINA: FORMULATE A NATIONAL ACTION PLAN

We suggest that the Chinese Government should make its „Action Plan for Rare Diseases“ as soon as possible to create an environment that promotes sustainable innovation and development. This will be a 5-year plan based on medical security, industrial development, laws, regulations and policies of rare diseases.

- I. Set up an individual Office of Orphan Medicines under the National Center for Drug Evaluation
- II. Prioritize the import of the innovative drugs for the rare diseases that can be diagnosed and treated
- III. Set up the National Rare Diseases Expert Committee under NHSA
- IV. Set up a national special fund for the medical security of rare diseases
- V. Give priority in addressing the accessibility problem of orphan medicines



- VI. Set up the annual upper limit for self-payment of treatment costs by rare disease patients <RMB 80,000
- VII. Improve the mechanism of updating the 121 Catalogue
- VIII. Support the innovation, R&D and manufacturing of native orphan medicines
- IX. Gradually set up national and regional rare disease diagnosis and treatment centers
- X. Strengthen the participation of patients and patient organizations in rare disease affairs