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 totall 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 DISEASE’
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 Shenzhen. The experts asserted that rare diseases should be defined as diseases with an incidence of less than 0.05/10,000 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 AS DILEMMA OF DRUGS BEING UNAVAILABLE
Rare disease drugs appeared in the past two round NDRD (National Reimbursement Drug List) national negotiations in China. Out of the 15 drugs, that are now available in China, and have rare disease indications, 12 have been included in the NDRD including 10 rare diseases. Among the 79 drugs, 42 are on the List. Without financial coverage 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 NDRD, 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 NDRD, 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 2003 Shanghai, Tongji and Zhongshan 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 some extent. Following table summarizes the key points of these Major Local Pilots for Rare Diseases Healthcare Security in China.

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 nd 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 too hard to be diagnosed; there are no medical products available to treat the diseases effectively; the treatment drugs are too expensive and 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 an ‘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 on 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 NSHA
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