

## Network established to collaborate on diagnosis and treatment of rare diseases in China: A strategic alliance backed by tiered healthcare is the key to the future

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### Summary

Rare diseases are an important public health issue and a challenge to healthcare. Over the past few years, China has actively worked to improve rare disease care and orphan drugs for rare diseases, but many challenges still remain. In order to further promote measures to combat rare diseases, the "National Network to Collaborate on Diagnosis and Treatment of Rare Diseases" was established by the National Health Commission of the People's Republic of China on February 12, 2019. This network for collaboration consists of 324 hospitals nationwide, and its aim is to set up a practical mechanism for an effective alliance by different tiers of the healthcare system. The strategy for collaboration includes six programs: *i*) to establish a mechanism for collaboration, *ii*) to implement standards of care, *iii*) to enhance quality control, *iv*) to ensure the supply of drugs, *v*) to set up a registry, and *vi*) to enhance clinical research. These programs will play a pivotal role in combating rare diseases in the future and eventually achieving the goal of creating a proper and consistent mechanism to treat and manage rare diseases.

**Keywords:** Rare diseases, network for collaboration, tiered healthcare

On February 12, 2019, establishment of the "National Network to Collaborate on Diagnosis and Treatment of Rare Diseases" was announced by the National Health Commission of the People's Republic of China to further combat rare diseases (1). The network consists of 324 hospitals nationwide, including 1 leading national institution, 32 leading provincial institutions, and 291 member institutions in accordance with recommendations from provincial health authorities and a panel of experts.

Worldwide, there are between 6,000 and 8,000 rare diseases. Eighty percent of rare diseases have identified genetic origins, 75% of rare diseases affect children, and 30% of patients with rare diseases die before the age of 5 (2,3). Rare diseases are serious chronic diseases and may be life-threatening. The features of rare diseases and the increasing number of identified

rare diseases make these diseases a priority for policymakers, researchers, legislators, and healthcare professionals (4,5).

Over the past few years, China has actively worked to improve rare disease care and orphan drugs for rare diseases, but it still lags far behind the US, EU, Japan, and other countries and regions in scientific research, diagnosis and treatment, protection of patient rights, public awareness, and other areas. The good news is that some provinces and cities in China have taken a step forward in policy-making and alliance building. Supporting measures have mainly been implemented by professional groups, such as the Specialty Committee of Rare Diseases of the Shanghai Medical Association since 2011 (6), the Shanghai Center for Diagnosis and Treatment of Rare Diseases, the Shanghai Children's Center for Diagnosis and Treatment of Rare Diseases, and the Shanghai Clinic Specializing in Rare Diseases established in 2018 (7), and the China Rare Disease Alliance consisting of more than 50 entities ranging from medical facilities, universities, academic institutions, and companies since 2018 (8). These measures are intended to promote research and

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development on rare diseases and orphan drugs.

On May 22, 2018, China's First National List of Rare Diseases, which includes 121 rare diseases, was jointly issued by five national bodies, including the National Health Commission, the Ministry of Science and Technology, the Ministry of Industry and Information Technology, the State Drug Administration, and the State Administration of Traditional Chinese Medicine (9,10). This is a historical breakthrough indicating that rare diseases are garnering more attention and that those diseases are being considered as part of national health strategy and planning. Given this situation, the "National Network to Collaborate on Diagnosis and Treatment of Rare Diseases" was approved as an important platform in February 2019. The goal of the Network is to further flesh out practical mechanisms and infrastructure to combat rare diseases in light of changing social and financial resources.

In order to facilitate coordination among the different tiers of the healthcare system, the "National Network to Collaborate on Diagnosis and Treatment of Rare Diseases" has clearly described workflow management and identified future strategies to deal with the challenge of rare diseases from various perspectives. Priorities are to establish a network for diagnosis and treatment of rare diseases, to create a comprehensive mechanism for coordination and designation of certain hospitals as bases for treatment through referrals in the tiered healthcare system, to eventually achieve the goal of early detection and diagnosis, and to create fair and consistent mechanisms to treat and manage rare diseases. The strategy for collaboration includes six programs. One is *i*) to establish a mechanism for collaboration. Leading hospitals and member hospitals will form a network to cover the screening, diagnosis, treatment, rehabilitation, and long-term management. The second program is *ii*) to implement standards of care. Clinicians will be trained to follow technical guidelines and a referral system will be developed to improve the rate of early diagnosis and treatment. The third program is *iii*) to enhance quality control. Provincial healthcare authorities will enhance management, guidance, and evaluation. Member hospitals will improve their diagnosis and treatment capabilities with an emphasis on safety and multi-disciplinary treatment (MDT). The fourth program is *iv*) to ensure the supply of drugs. Member hospitals will properly manage orphan drugs and fulfill clinical demand. The supply chain will make picking up drugs more convenient for patients with rare diseases. The fifth program is *v*) to set up a registry. The National Health Commission has set up a registry for rare diseases. Member hospitals will collect data and statistics. The sixth program is *vi*) to enhance clinical research. Basic,

clinical, and translational research will be encouraged, and international exchanges and cooperation will be enhanced.

Rare diseases are a pressing public health issue and challenge to healthcare worldwide. The kickoff of the "National Network to Collaborate on Diagnosis and Treatment of Rare Diseases" represents a positive step by the government to formulate measures to combat rare diseases in China, and this effort portends a number of efforts in the future. Substantial efforts are urgently needed, and society is still calling for the enactment of legislation and accompanying regulations on rare diseases and orphan drugs.

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