HEALTH FOR ALL

China’s Initiative to Combat the Challenges of Rare Diseases

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‘Health is a prerequisite for people's all-round development and a precondition for economic and social development.’

— President Xi Jinping, National Health Conference, 2016
Policies at National Level for Rare Diseases Care in China

1. Policy Making and the Consulting Organization
2. Clinical Care System governed by China NHC
3. National Rare Diseases Registry System supported by PUMCH
4. Specialty Communities, Workforce Training and Medical Education
5. Prioritized Approval Process by China FDA
Policies at National Level for Rare Diseases Care in China

6. Tax reduction for Orphan Drugs by Central Government

7. Research Funding in Rare Diseases

8. Social Care and Charities

9. Broad Involvement of Multiple Stakeholders

10. Collaboration with International Communities
2016.1 Establishment of rare diseases experts committee
(Diagnosis/Treatment/Medical security) (Member of National Health committee)

Primary task:
Catalogue of Rare Diseases in China
China’s First List of Rare Diseases (2018.5)

Five ministries jointly issued the first version of RD catalogue
121 diseases included
The second edition will be updated soon
A Nation-wide Medical Care System

324 hospitals designated as the Clinical Center of Rare Diseases

National (1) PUMCH
Provincial (32), Municipal (291)

Directed by the National Committee of Health
(equivalent to China Ministry of Health)
Duties of Medical Care System

1. Screening and Primary Diagnosis
2. Dual-Direction Referral of Undiagnosed Patients
3. Long-term Clinical Management
4. Consulting for Child-birth
5. Report and Registry
6. Priority for Orphan Drug Distribution
7. Clinical Trials of Orphan Drugs
National Rare Diseases Registry System of China

Initiated from Dec, 2016

Supported by
① National Health Committee, China
② Ministry of Science and Technology, China
③ Peking Union Medical College Hospital
④ Total budget: 40,000,000 RMB

www.nrdrs.org.cn
National Rare Diseases Registry System

Statistics

1. Over 30,000 Cases (Clinical + Bio samples)
2. Over 180 Disease Entities
3. > 150 Experts
4. > 50 Research Institutes

Long-term Goal

1. Phenotypic-Genomic Integrated Data Platform
2. Research Infrastructure for China and the World
3. Accurate Statistics of RD in China
Associations and Workforce Training

The Rare Diseases Research Center of Chinese Academy of Medical Sciences (CAMS)

The Rare Diseases Society of the Chinese Association of Research Hospitals

Provincial Rare Disease association

1. Beijing
2. Shanghai
3. Shandong
4. Others

Nation-wide Training of the Clinical Care and Research of Rare Diseases
Medical Education — Publication

Compendium of China’s First List of Rare Diseases

Handbook for the Care of Rare Diseases
Publishing Special Issues on Rare Diseases and Orphan Drugs
Publishing Special Issues on Rare Diseases and Orphan Drugs

National Rare Diseases Registry System of China and Related Cohort Studies: Vision and Roadmap

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Introduction
Rare diseases affect a large group of health-related services without a universally recognized definition. The definition of rare diseases as a whole varies by country and according to specific environmental circumstances. For example, the United States defines rare diseases as disorders affecting <200,000 individuals, while the European definition is disease with prevalence of <10,000 people. Despite the low prevalence of a given rare disease, the overall population affected by rare diseases in general is strikingly large due to the great variety that ratio. Rare diseases are substantially contributed to a high disease burden for patients, families, and communities around the world.

"Considering the vast number of people with rare diseases in the world, rare diseases and public health are not an oxymoron. They are a real phenomenon that should be considered as a world-health issue". Multiple rare diseases are associated with chronic disease syndromes, chronic disease burden, and economic costs.

Keywords: rare diseases, patient registry, cohort study, medical information, insurance

Exporting to Excel

National Rare Diseases Registry System of China Vision and Roadmap

The National Rare Diseases Registry System of China (NRDS) is an information platform that is expected to accelerate integration of clinical diagnosis and treatment information with the data and sample library of clinical cohort studies in order to identify accurate approaches to the diagnostic and treatment of rare diseases. Indeed, the study of disease largely simplifies to diagnosis and makes early intervention possible, especially for rare diseases with common clinical manifestations.

The NRDS plans to conduct cohort studies of 300,000 cases of at least 50 types of rare diseases. The list consists of cardiovascular, pulmonary, urinary, endocrine, metabolic, hematologic, oncologic, musculoskeletal, and dermatologic diseases. For patients suspected of having one of the above diseases, it is necessary to collect and analyze specific clinical data and research information that are unique to traditional etiologic diagnosis.

The main topics covered in the registry include the general clinical data, the specialty-specific clinical data, and the laboratory test results. These data will be used to establish a clinical database for noncommunicable diseases, which will be integrated into the NRDS. Finally, the database will be used to generate a detailed rare disease classification and to identify patient treatment models.

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Other Medical Education programs

• Translation of Gene Reviews into Chinese by NRDRS
  (7 million characters in total)

• Initiation of the Textbook of Rare Diseases for graduate students

• Collaboration with different specialty societies for Continued Medical Education
Rare Disease Medication Dilemma

- Diseases without any effective drug or treatment
- Effective drugs are not available in China
- Effective drugs are available but no indication of RD
- Most of RD Drugs are unaffordable and not in the medical secure list

**NEED TO IMPROVE:**

- Orphan Drug Research and Development
- Approaches to Drug Import
- Clinical Trials
- Medical Insurance Policy
1. Fast-track for drug approval for the orphan drugs approved in the US, EU and Japan
2. China has formed an approval process for orphan drugs in urgent need
3. Criteria
   ① With indication for the entities enlisted in the First List of Rare Diseases
   ② Drugs for severe and life-threatening conditions that has no effective therapeutic methods
   ③ Drugs for severe and life-threatening conditions and those with significantly increased clinical benefit.
4. Approval Duration: 3-6 months
5. Post-marketing Research requested for efficacy and safety evaluation
6. The System of Health Technology Assessment (HTA/HEOR) for Rare Diseases and Orphan Drugs
Tax Reduction for Orphan Drugs

1. Announced on Feb 11th by Prime Minister Li Keqiang

2. Value-added tax 16% → 3% (80% Reduction)

3. 21 Orphan Drugs

4. March 1st 2019

5. Perspectives

① Reduction of the financial burden of patients

② Incentive for orphan drug pharmaceutical companies

③ Promotion of the social awareness of rare diseases and orphan drugs
Research Funding

1. National Key Science and Technology Research Program
   ① Rare Diseases Registry and Cohort Studies > 40m RMB
   ② Diagnostics Technologies Innovation > 20m RMB
   ③ Other related Diseases > 50m RMB

2. National Natural Science Foundation, Special Fund for Rare Diseases

3. Provincial Level Research Funding for Rare Diseases
   ① Beijing  ② Shanghai  ③ Guangdong  ④ Zhejiang  ⑤ Shandong

4. Novel Drug Discovery and Development Program
Social Care and Charities

1. Regional Social Insurance Coverage
   ① Qingdao
   ② Shanghai
   ③ Beijing
   ④ others

2. Disability Care and Support

3. Rehabilitation and Enforcement of Employment

4. Prohibition and Prevention of Discrimination

5. Financial Support Program by Various Charities
Broad Involvement of Multiple Stakeholders

International Rare Diseases Day 2018
The Rare Disease Drug Development Forum-PUMCH
Broad Involvement of Multiple Stakeholders

2018.10
China Alliance For Rare Diseases

Academic organization
Research Institute

Pharmaceutical enterprises
Hospital

Patient group
Public funds
Media publicity

Government
Members of China Alliance For Rare Diseases

- Hospitals: 48 from 26 provinces
- Enterprises: 36 (Biotechnology, Information Technology, and Pharmaceutical enterprises)
- Government departments: 3
- Academic organization: 5
- Research Institutions: 2
- Patient group: 2
- Public funds: 1
- Media: 1
Duties of China Alliance For Rare Diseases

- Research on policy
- Academic communication
- Shared data platform
- Standards for medical care

Innovation of orphan drugs

Rapid Product translation
Professional training
International cooperation
Social public service
Collaboration with International Communities

- ICORD
- Orphanet
- IRDiRC
- SNOMED International
- AMIA
- Broad Institute
- Yale University
- Weizmann Institute of Science
Welcome to NCCCORD

National Conference of China on Rare Diseases and Orphan Drugs
RARE DISEASES IN CHINA:
TOWARDS A BETTER AND FAIRER FUTURE
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- Project Leader, National Rare Diseases Registry System of China
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