The Urgent Need to Empower Rare Disease Organizations in China: An Interview-Based Study

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Research

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Abstract

Background: Each rare disease affects a small number of population, and thus is neglected. However, a total of 7,000 rare diseases may affect 10% of the population. Due to the severity and lack of rare disease awareness, rare disease represents a huge challenge for the healthcare system. In Western countries, patient organizations have been playing an integral role in raising awareness, advocating legislation, and supporting drug development. This study aims to assess the unmet needs of rare disease patient organizations in China, and identify their unmet needs, providing essential information for the government and legislators.

Results: A total of 28 individuals representing 28 patient organizations in China were interviewed. Most organizations do not have official registration, employees, written standard operation protocol, or reliable financial resources. Misdiagnosis or delayed diagnosis is common, and treatment is often lacking. Due to the lack of financial resources, no organizations have been able to sponsor academic research, unlike their counterparts in Western countries. As to challenges, 71.4% of interviewees listed lack of rare disease awareness among the general public, while 67.9% selected lack of financial resources. Further, only 7.3% of these organizations received support from the government, and 28.6% received support from the general public. As to recommendations to the government, 82.1% of interviewees selected special insurance programs for rare diseases because rare diseases have been generally excluded from the national medical insurance programs. In addition, 78.6% of interviewees recommended to stimulate rare disease research, 75% recommended to import orphan drugs, and 71.4% recommended legislation of an orphan drug act, highlighting the urgent need of therapies.

Conclusions: Due to lack of support and rare disease awareness, patient organizations in China are still in the early phase. To empower these patient organizations, recommendations from these patient organizations should be considered by the government and legislators.

Background

Rare diseases are diseases that affect a small number of population. Most rare diseases have identified genetic origins, and 50% of patients with rare diseases die before the age of 5 [1]. It was estimated that a total of 7,000 rare diseases affect 300 million people worldwide. The definition of rare diseases differs country by country. In the United States, a rare disease is defined as any disease with a prevalence of fewer than 200,000 patients. The equivalent number in Japan is 50,000 patients, while in the European Union, the definition is any disease that affects 1 in 2,000 people. Although rare diseases vary in etiology and clinical manifestations, most are associated with significant disease burden impacting physical and mental abilities, as well as life expectancy [2, 3, 4]. As a huge group, rare diseases constitute a significant challenge for the healthcare system and the economy, and thus should not be neglected by the society and the government [5, 6].

Patient organizations have been playing a critical role in forming supportive groups, advocating to reduce health disparity and discrimination, as well as building a sense of community [7]. Moreover, patient organizations are the driving force of the rare disease ecosystem and have a role at all levels, including supporting patients, initiating and sponsoring research, prompting legislation, generating educational materials, and raising public awareness [8]. Due to various reasons, the rare disease ecosystem in China is still underdeveloped. It is essential to evaluate the status quo of patient organizations, a key player in the ecosystem, and identify their unmet needs and ways to empower them. Therefore, an interview-based study was conducted, and recommendations were made for consideration of policymakers.

Results

Information about patient organizations

How interviewees got involved with a rare disease

A total of 28 interviewees, representing 28 patient organizations, participated in this study. Information about the 28 interviewees and their organizations is summarized in Table 1. Most (25/28, 89.3%) interviewees got involved because they or their family members had a rare disease. Twelve out of 26 respondents (46.1%) were funders of these patient organizations. Two organizations were started by individuals who were not personally affected by rare diseases.
| Organizations                                | Disease of interest                        | Relationship to a rare disease? | Is the interviewee the founder? | Current members | Total patients in China | Reliable financial resource | Official registration? | Employee? | Office space |
|---------------------------------------------|-------------------------------------------|--------------------------------|---------------------------------|-----------------|------------------------|----------------------------|------------------------|-----------|-------------|
| Anning's Mother PKU Chat Group              | Phenylketonuria                           | Family                         | Yes                             | 1,000           | 140,000                | No                         | No                     | No        | No          |
| MPS I Chinese Patients Community            | Mucopolysaccharidosis type I              | Self                           | Yes                             | Not sure        | Not sure               | No                         | No                     | No        | No          |
| Cushing Syndrome Community                  | Cushing syndrome                          | Self                           | Yes                             | 800             | 4,000                  | No                         | No                     | No        | No          |
| Nanjing Rare Disease Help Center            | Acromegaly                                | No                             | Yes                             | 800             | 100,000                | No                         | Yes                    | No        | No          |
| Usher Syndrome Chat Group                   | Usher syndrome                            | Self                           | No                              | Not sure        | 30,000–40,000          | No                         | No                     | No        | No          |
| Beijing Zhi'ai DMD Help Center              | Duchenne muscular dystrophy               | Family                         | No                              | 5,000           | 70,000                 | No                         | Yes                    | Yes       | No          |
| Sichuan Huntington's Disease Community      | Huntington's disease                      | Self                           | No                              | 600             | 4,000–5,000            | No                         | No                     | No        | No          |
| Gaucher Disease Patient Club                | Gaucher disease                           | Self                           | No                              | 300             | 400                    | No                         | No                     | No        | No          |
| Body Odor Chat Group                        | Unidentified diseases that cause body odor| Self                           | Yes                             | 2,000           | 20,000–50,000          | No                         | No                     | No        | No          |
| Acromegaly Communication Center             | Acromegaly                                | Self                           | Yes                             | 600             | 100,000                | NO                         | No                     | Yes       | No          |
| Shanghai ALD Mutual Help Group              | Adrenoleukodystrophy                      | Family                         | No                              | 1000            | Not sure               | No                         | No                     | No        | No          |
| CAH & AHC Help Center                       | Congenital adrenal hyperplasia, adrenal hypoplasia congenita | Family | Yes | 10,000 | 100,000 | No | No | No | No |
| SPE Patients Chat Group                     | Symmetrical progressive erythrokeratoderma | Family | No | Not sure | 1,000–10,000 | No | No | No | No |
| VWD Patient Community                       | Von Willebrand disease                    | Self                           | Yes                             | 200             | 160,000                | No                         | No                     | No        | No          |
| CGL Patient Community                       | Chronic Granulocytic Leukemia             | Self                           | Yes                             | Not sure        | Not sure               | No                         | No                     | No        | No          |
| LCA Patients Club                           | Leber congenital amaurosis                | Self                           | No                              | 300             | 30,000                 | No                         | No                     | No        | No          |
| Shandong Osteogenesis Imperfecta Chat Group | Osteogenesis imperfecta                   | Self                           | No                              | 600             | Not sure               | No                         | No                     | No        | No          |
| Pompe Patients Help Center                  | Pompe disease                             | Self                           | No                              | 400             | 5,000                  | No                         | No                     | Yes       | No          |
| LNS-China                                   | Lesch–Nyhan syndrome                      | Family                         | Yes                             | 15              | 200                    | No                         | No                     | Yes       | No          |
| Chinese Fabry Patients Club                 | Fabry disease                             | Self                           | No                              | 300             | 1,000                  | No                         | No                     | No        | No          |
| Zhuo Wei Chang Dao                          | Dravet syndrome                           | Family                         | Yes                             | 1,000           | 20,000                 | No                         | No                     | No        | No          |
| Beijing Zhengyu MPS Disease Center          | Mucopolysaccharidoses                     | Family                         | No                              | 400             | 2,000–3,000            | No                         | No                     | No        | Yes         |

SOP, standard operation protocol.
Some corporations in the USA have a special team to donate when approaching the tax filing date so that they can get a tax cut. Another interviewee mentioned that ‘Like China, many patient organizations in the USA received donations from corporations, pharmaceutical or other industries, because these corporations would receive tax benefits.’ Another interviewee mentioned that ‘Some corporations in the USA have a special team to donate when approaching the tax filing date so that they can get a tax cut.’
Rare diseases

Diagnosis

Genetic testing is largely unavailable or unaffordable, and there lacks genetic counseling. According to these interviewees, patients and families often receive a report full of jargon and technical terms without sufficient explanations or firm conclusions. It usually turns out that ‘the genetic test report generated more questions than it answered.’ All interviewees expressed concern over misdiagnosis and delayed diagnosis. Newborn screening was believed to be essential, and some interviewees initiated campaigns to implement newborn screening programs in China. Through one interviewee’s efforts, MPS I was included in a newborn screening panel in several hospitals in Beijing. She commented that ‘Although national newborn screening is not realistic now, this is an initial step.’

Treatment and management

A previous study showed that most rare disease patients had experienced difficulty in access to treatment, and fewer than 10% have received disease-specific treatment [1]. None of the rare diseases associated with these organizations are curable, only 10 (37.0%) have disease-specific treatment. Even when there is an option, the delayed diagnosis or misdiagnosis would have already costed patients the opportunities to receive timely treatment. For instance, MPS I disease can be treated by stem cell transplantation, which can halt neurological deterioration and improve quality of life if performed before age 2. One organization, Seven-Pansy had been trying to import orphan drugs through a government-sponsored special project called Hainan Boao Lecheng International Medical Tourism Pilot Zone [17]. This project aims to import drugs due to urgent clinical needs for us in designated medical institutions. Based on patients’ needs, Seven-Pansy had drafted a list of orphan drugs for the Boao project.

Activities

Rare disease awareness

Large organizations in Western countries, for instance, the National Organization for Rare Disorders (NORD) in the United States and the European Organization for Rare Diseases (EURORDIS) in Europe, have been contributing significantly to raising rare disease awareness. The Rare Disease Day event, initiated by EURORDIS, has now become a well-known event worldwide. However, such a national platform or information hub with a similar influence still lacks in China. As shown in Fig. 1A, 23 of 28 organizations (82.1%) had activities to raise rare disease awareness, mainly through the internet. Several interviewees mentioned that they had organized members to hand out pamphlets on the street. There has been increasing rare disease awareness in China [9, 18], which may be attributed to the efforts of patient organizations.

Patient support

Since most of these organizations had no reliable funding source, only 4 organizations (14.3%) were able to provide financial support to patients and families. A total of 24 organizations (85.7%) provided education materials with input from physicians and researchers. However, none of these organizations were able to generate educational materials to guide physicians, which have been attempted by organizations in Western countries. For instance, NORD issued a comprehensive physician guide to rare diseases [8]. Most organizations (20/28, 71.4%) provided consulting services to patients and families. This was mainly through expert patients or occasionally through invited physicians. Another form of patient support is local meetup, which had been performed by 13 organizations (46.4%). Three interviewees commented that local meetups could provide more direct communication, which is important to relieve stress and seek comfort from each other.

Research

In Western countries, many patient organizations sponsor academic research with the main focus on therapy development [19, 20]. For instance, many organizations, such as NORD (USA), Association Française contre les Myopathies (France), Children Living with Inherited Metabolic Disorders (UK), Sanfilippo Children’s Foundation (Australia), have annual grant programs to support rare disease research. Also, several patient organizations in Europe, Australia, and the United States contributed millions of dollars and helped ABO-101 and ABO-102, two gene therapy products for MPS III diseases, reach the clinical trial stage. In contrast, patient organizations in China, mainly due to the lack of financial support, have been not able to sponsor academic research. Out of these 28 organizations, 5 (17.9%) had helped to recruit patients for clinical trials, and 13 (46.4%) maintained patient registries. Although it is essential to have a national registry for rare diseases, these organizations have not been able to establish one. In Western countries, there also has been a transition of patients as participants or financial supports into collaborators in research. Members of patient organizations have been actively involved in academic research and drug development, resulting in peer-reviewed publications and patents [21, 22]. This is rarely seen in China, and none of the interviewees in this study had played such a role in academic research.

Unmet needs of patient organizations

Challenges

Most organizations (20/28, 71.4%) listed the lack of rare disease awareness among the general public as a major challenge. Also, 19 organizations (67.9%) listed the lack of financial sources as a major challenge. Fifteen organizations (53.6%) believed contacting other patients was challenging, 14 (50%) selected communication with doctors, while 14 (50%) selected the lack of reliable information source (Fig. 1B). Three interviewees mentioned that the availability and affordability of orphan drugs were poor.
Support received

Only 1 (3.6%) received support from the central government, and only 2 received support from the local governments (Fig. 1C). Only 8 (28.6%) received support from the general public. The major source of support these organizations received came from non-profit organizations (21/28, 75%), pharmaceutical companies (14/28, 50%), and hospitals (15/28, 53.6%). These results indicated a significant lack of support from the government and the general public.

Interviewees’ recommendations to the government: As shown in Fig. 1D, 23 interviewees (82.1%) selected ‘special insurance program for rare diseases or inclusion of rare diseases into the Critical Illness Insurance Program’. Twenty-two interviewees (78.6%) selected ‘stimulate rare disease research’, 21 (75%) selected ‘import more orphan drugs’, and 21 (75%) selected ‘establish a platform to provide reliable information’. Also, 20 interviewees (71.4%) selected ‘legislate orphan drug act’, 17 (60.7%) selected ‘provide financial support to patients’, and 15 (53.6%) selected ‘provide financial support to patient organizations’. Additionally, 14 (50%) selected ‘address discrimination in school’, 10 (35.7%) selected ‘simplify registration process of organizations’, and 2 (7.1%) selected ‘disability certificate’. One interviewee commented that ‘raise public awareness so that patients can be respected, understood, and equally treated by employers’. He further explained: ‘some local governments had written policies to exclude patients with acromegaly while hiring civil servants.’ This practice of the local governments is directly against the Law of the People's Republic of China on the Protection of Disabled Persons [23].

Discussion

Patient organizations have been playing a critical role in advancing the field of rare diseases by advocating patient’s voices and addressing the unmet needs of patients. In the 1980s, the NORD organized a series of headline events that raised wide public awareness and prompted the legislation of the world’s first orphan drug act [24, 25]. This act provides incentives, including market exclusivity, tax benefits, fast track approval, and government subsidy, to stimulate orphan drug development. These incentives have been to be particularly appealing to pharmaceutical companies [26, 27]. The number of orphan drugs increased from 38 in 1983 to over 500 in 2018. Therefore, similar acts have been established in many other countries and districts, including Japan, European Union, Singapore, South Korea, and Australia [28, 29]. EURODIS and Canadian Organization for Rare Diseases (CORD) also have been playing an integral role in spearheading rare disease and orphan drug legislation, patient support, raising awareness, and sponsoring academic research.

Compared with patient organizations in the United States, Europe [30], Australia [31], and India [32], patient organizations in China are still in the early phase. This is mainly due to the lack of support from the government and rare disease awareness among the public. This study identified key challenges and unmet needs of patient organizations in China. To empower patient organizations in China and thus advance this field, key recommendations that outline top priorities that the government and legislators should consider are listed as follows.

1. Legislate an Orphan Drug Act to stimulate orphan drug development (orphan drug exclusivity, tax incentives, market exclusivity).
2. Issue official definition of rare disease.
3. Raise rare disease awareness to shorten the diagnosis odyssey.
4. Implement prenatal and newborn screening of a subset of rare diseases.
5. Establish special insurance programs to cover the cost of the treatment and long-term management of rare diseases.
6. Issue regulations to protect rare disease patients from discrimination in education and employment.
7. Import more orphan drugs.
8. Stimulate donations to rare diseases by tax deduction or other types of tax benefits for individuals and corporates.

Conclusions

Rare disease patient organizations have been playing a critical role in advocating patients’ voice, prompting legislation, raising awareness, educating and supporting patients, and sponsoring academic research. However, unlike their counterparts in Western countries, patient organizations are less developed due to lack of support and rare disease awareness among the public. This study evaluated the current status of 28 patient organizations through an interview-based study and identified their unmet needs. Recommendations have been made for the policymakers to consider to empower patient organizations in China.

Methods

Ethics, consent, and permission

The study was approved by the Institutional Ethics Committee of Guangzhou Medical University. Potential interviewees were invited to participate this study, and only those who signed the informed consent participated in this study. All the interviewees acknowledged: 1) the affiliation of the investigators; 2) the sponsor of the study; 3) the objectives of the study, 4) that the information collected will only be used for academic research; 5) that they will participate in this study anonymously; 6) that they can decline to answer any of the questions; 7) that they can quit the study at any time; 8) that the results will be published in a scientific journal without seeking their approval of the manuscript; and 9) that they will not be paid for participating in this study.

Data collection and analysis

The interviewees, usually a key person in a patient organization, were recruited through online advertisements. An online webinar was held to brief potential participants on the background of the study and answer their questions. Then, the investigators contacted potential participants to confirm the interests and schedule the interview. Finally, the interviews were performed via a 1 to 2-hour phone talk. All interviews were conducted by the investigators between March to May 2020. The interviews were audio-recorded with the interviewees’ permission and transcribed verbatim for further analysis.
Abbreviations

National Organization for Rare Disorders (NORD), European Organization for Rare Diseases (EURORDIS), Canadian Organization for Rare Diseases (CORD)

Declarations

Ethics approval and consent to participate

The study was approved by the Institutional Ethics Committee of the Guangzhou Medical University, China. All interviewees signed the informed consent and agreed to participated this study voluntarily.

Consent for publication

Not applicable

Availability of data and materials

The interview transcripts are not publicly available due to the agreement between the investigators and the interviewees.

Competing interests

The authors declare that they have no competing interests.

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Authors’ contributions

XL, LO, and SZ designed the study. ZL and JZ performed interviews and data analysis. XZ, BL, JZ performed statistical analysis. LO wrote the manuscript.

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Figures
Activities, challenges, support received, and recommendations of patient organizations. Activities (A), challenges (B), support received (C), and recommendations (D) were shown, respectively. Information was collected through one-on-one interview with leaders from 28 patient organizations in China.