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Original article

Current care practices for patients with Duchenne muscular dystrophy in China

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Abstract

Background: The coronavirus disease (COVID-19) pandemic has presented challenges in the care of patients with chronic diseases. We identified the challenges faced by Chinese patients with Duchenne muscular dystrophy (DMD) during the pandemic.

Methods: An online cross-sectional survey was conducted between March 27 and June 30, 2021.

Results: Of the 2105 valid questionnaire responses, 2,056 patients were from non-lockdown areas. In these areas, 42.8% reduced outside daily activities, 49.4% reduced rehabilitation service use, 39.7% postponed regular follow-ups, and 40.8% reported accelerated motor function decline. These figures generally increased for patients from lockdown areas—67.3% reduced outside daily activities, 44.9% reduced rehabilitation service use, 79.6% postponed regular follow-ups, and 55.1% reported accelerated motor function decline. Ambulation loss was most commonly reported in September and March before 2020; however, this trend was absent in 2020. Regarding the informed prices of disease-modifying drugs in Europe and the United States, 86.7% could afford a maximum of one-twentieth of the prices, 8.0% could afford one-tenth of the prices, and only 0.6% of the patients could afford the full prices.

Conclusions: Implementation of standardized care for DMD in China is consistent with global practices, and the COVID-19 pandemic has affected the care of patients with chronic diseases worldwide, particularly in lockdown areas. Telemedicine is an effective model for providing healthcare to such patients. Healthcare workers should assist patients and establish more robust chronic disease management systems. Collaboration between governmental and non-governmental entities could address the cost of disease-modifying drugs in China and other developing countries.
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Keywords: Duchenne muscular dystrophy; Public health emergency; COVID-19; Care considerations; Disease-modifying treatment

1. Introduction

Duchenne muscular dystrophy (DMD) is an X-linked neuromuscular disorder affecting 1/5000–6000 live male births caused by mutations in the dystrophin gene located at XP21.2–21.1. It is characterized by progressive muscle weakness in its first stage (i.e., early childhood) and multisystemic involvement in its second

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stage (i.e., early adolescence). Cardiomyopathy, respiratory failure, or both develop in DMD's third stage (i.e., the teenage years) and cause early death [1]. Although DMD is currently incurable, multidisciplinary interventions such as corticosteroid therapy, rehabilitation, cardiac management, and ventilation support are feasible methods for improving patients' quality of life and extending their survival. Corticosteroids are the current standard therapy for patients with DMD, but long-term steroid use can result in cushingoid appearance, behavior changes, growth delay, low bone mass density and/or fracture, cataracts, etc. [2]. Meanwhile, respiratory muscle weakness in patients with DMD can cause weak cough, atelectasis, pneumonia, and respiratory failure [3].

Since late 2019, the coronavirus disease (COVID-19) outbreak has become a global public health emergency, and patients with DMD are classified as a group at high risk of morbidity and mortality due to COVID-19 [4]. To better understand the challenges faced by patients with DMD in China during the pandemic, we conducted a cross-sectional survey. Such research involving DMD patients in China is rare.

2. Methods

2.1. Questionnaire design

We conducted a cross-sectional survey of patients with DMD through a Chinese questionnaire site (https://www.wjx.cn, article in Chinese) and a social media platform called WeChat. The survey comprised three main sections with a total of 30 questions. The first section concerned general demographic and clinical information, including age, domestic address(es), DMD-causing mutations, glucocorticoid regimens, and ambulatory status. The second section focused mainly on how the public health emergency control measures had influenced the care and treatment of patients. The third section concerned patients' access to medical care, including essential medications and new disease-modifying drugs.

All patients with DMD in our national registry database or WeChat groups were invited to complete the survey. The questionnaire was available between March 27 and June 30, 2021.

Definitions: Loss of independent ambulation (LoA) or non-ambulation was defined as the initiation of continuous wheelchair use [2]. "Reduced activity" in this study was defined as a reduction in usual activity after the pandemic by more than a third of that before the pandemic. "Decline of motor function" was defined as an increase in fall times of the patient after the pandemic by more than one-third of that before the pandemic; or an increase of one-third in the time it takes to ascend and descend stairs than before the pandemic; or a decrease in the patient's walking speed by more than a third of the speed before the pandemic. "Expectance for more assistance from the society" was assessed by the following question in our questionnaire, "Do the patients require more social support after the pandemic than before the pandemic? Such patients were those with difficulty in gaining rehabilitation knowledge and guidance, difficulty in acquiring assistive rehabilitation devices or drugs, and financial difficulties in household income reduction during the pandemic period.".

2.2. Data analysis

Group descriptive statistics were expressed as means and grouped frequencies. We used the χ^2 test or Fisher's exact test for between-group comparisons. We conducted all data analyses with Prism GraphPad software (version 8.0; GraphPad Software, San Diego, California, USA). Statistical significance was set at $p < 0.05$.

2.3. Ethics

Our institutional ethics committee approved this study (No. KY2121-001). Online informed consent was obtained from the patients or their parents/caregivers before the start of the survey.

3. Results

3.1. Respondent profiles

We obtained valid questionnaire responses from 2105 patients with DMD, of whom 2.3% (n = 49) lived in areas subject to pandemic-related lockdowns. All participants in this survey are male. The 2,056 respondents in non-lockdown areas had a mean age of 8.7 years (range, 0.4–28.9 years). The mean age at diagnosis was 4.3 years (range, 0.1–12.5 years). Of the 1607 participants who were ≥5 years old and lived in non-lockdown areas, 1234 (70.8%) received corticosteroids, with 83% receiving prednisone/prednisolone and 17% receiving deflazacort. The 49 respondents in lockdown areas had a mean age of 8.7 years (range, 1.4–20.4 years), and the mean age at diagnosis was 3.6 years (range, 0.3–9.6 years). Corticosteroid treatments were received by 71.8% (28/39) of those who were ≥5 years old.

Of the 2105 respondents, 78.1% (n = 1646) remained independently ambulatory. Of the 459 non-ambulatory patients, 10.0% (n = 46) used part-time nocturnal non-invasive ventilation, and the remaining 90.0% (n = 392) never used assisted ventilation. The months in which the majority of patients experienced the loss of independent ambulation were September (n = 57) and March (n = 76), which are the months after the

summer and winter vacations, respectively, for Chinese students (Fig. 1). Among the 305 patients who experienced the loss of independent ambulation before 2020, this loss of ability was most commonly reported in September (n = 41) and March (n = 47). However, this trend was not noted for the 154 patients who lost independent ambulation ability in 2020 during the COVID-19 pandemic.

3.2. Impact of the COVID-19 pandemic on patients' care considerations

Of the respondents from non-lockdown areas, 42.8% (n = 880) reduced their outside daily activities such as walking, swimming, and cycling; 49.4% (n = 1,016) reduced their use of rehabilitation services; and 39.7% (n = 816) postponed their regular follow-up appointments. Furthermore, 40.8% (n = 838) complained of accelerated declines in motor function over the previous year (Fig. 2).

Changes due to COVID-19 were more pronounced for the respondents from lockdown areas. Of those respondents, 67.3% (n = 33) reduced their daily outside activities, 44.9% (n = 22) reduced their use of rehabilitation services, and 79.6% (n = 39) postponed their regular follow-up appointments. Furthermore, 55.1% (n = 27) reported an accelerated decline in motor function over the previous year. The *p* values for between-group differences are provided in Table 1.

Of the respondents from non-lockdown areas, 60.8% (n = 1,250) expected to receive more assistance from the society than they had received before the pandemic. Of the respondents from lockdown areas, 87.8% (n = 43) had the same expectation. When asked whether they felt more anxious than usual and needed psychological counseling, 11.5% (n = 236) of patients from non-lockdown areas and 18.4% (n = 9) of the patients from lockdown areas responded affirmatively.

3.3. Impact of the COVID-19 pandemic on patients' access to medical care

Of the respondents from non-lockdown areas, 76% (n = 1563) had at least one telemedicine visit, and 71% (n = 1109) of those who utilized telemedicine thought it could partially solve the problem of poor access to medical services for patients during the pandemic. Of the respondents from lockdown areas, 91.8% (n = 45) had at least one telemedicine visit, and 66.7% (n = 30) of those who utilized telemedicine reported that it was helpful for patients' access to medical services during the pandemic era. The question on the reasons for dissatisfaction with telemedicine was an open question. The two commonest reasons were lack of a physical examination and insufficient time for a satisfactory teleconsultation, when compared to face-to-face consultations.

Our survey results also showed that the price of newly approved DMD medications was a potential barrier to care. When our respondents' parents were informed of the prices of such medications in Europe and the United States, 86.7% (n = 1826) reported that they could afford a maximum of one-twentieth of the prices, 8.0% (n = 168) reported that they could afford one-tenth of the prices, and only 0.6% (n = 13) of patients could afford the full prices. Nevertheless, all of our patients desired information concerning the latest drugs for DMD approved by the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA), and 88.9% (n = 1871) of our respondents would like to participate in global clinical trials assessing new drugs for DMD if they are eligible to receive them.

4. Discussion

Standardized management strategies are crucial for patients with neuromuscular diseases such as DMD. Studies show that implementing standardized manage-

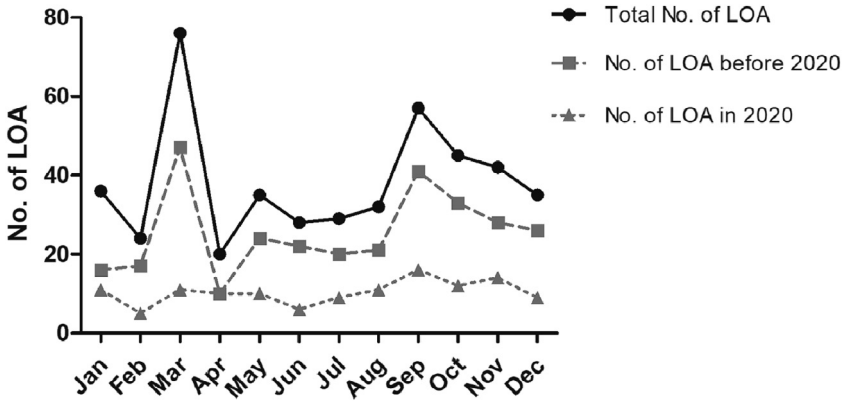

Fig. 1. Number of patients with DMD who experienced LOA during each month. LOA: loss of ambulation, DMD: Duchenne muscular dystrophy.

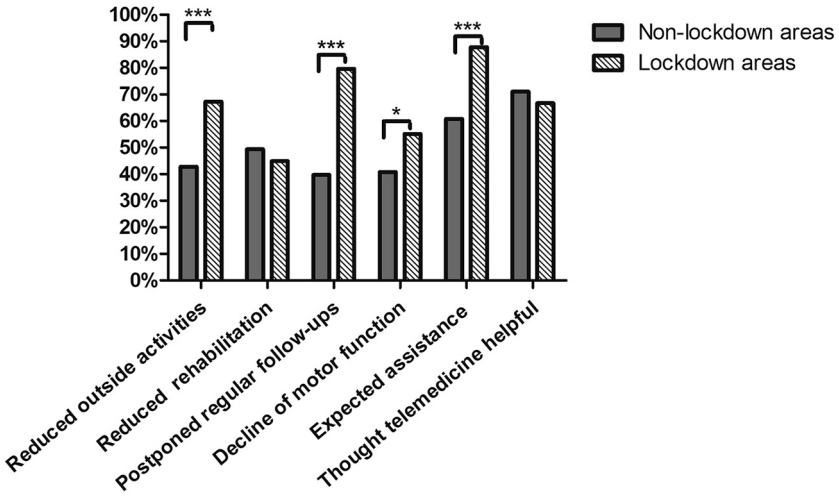

Fig. 2. Main influence of public health emergency control measures on healthcare for patients with DMD. DMD: Duchenne muscular dystrophy.

Table 1
Impact on activities and services provided for patients with DMD during a public health emergency.

Items	Participants in non-lockdown areas	Participants in lockdown areas	*P* value
Mean age (range)	8.7y (range: 0.4, 28.9 years)	8.7y (range: 1.4, 20.4 years)	_
Reduced outside activities	42.8% (880/2056)	67.3%(33/49)	0.001(***)
Reduced rehabilitation	49.4%(1016/2056)	44.90% (22/49)	0.532
Ever postponed regular clinical follow-ups	39.7%(816/2056)	79.6%(39/49)	<0.001(***)
Decline of motor function	40.8%(838/2056)	55.1%(27/49)	0.044 (*)
Ever used telemedicine	76% (1563/2056)	91.8% (45/49)	0.010 (**)
Thought telemedicine helpful	71% (1109/1563)	66.7% (30/45)	0.533
Expected more assistance	60.8% (1250/2056)	87.8%(43/49)	<0.001(***)
Need of psychological counseling	11.5%(236/2056)	18.4%(9/49)	0.137

DMD: Duchenne muscular dystrophy.

ment of DMD delays the loss of ambulation and extends overall survival [5,6]. As evidenced by the publication of national clinical care guidelines for DMD, standardized management for patients with DMD has been promoted and implemented in China. All respondents had visited a specialized neuromuscular center at least once. The mean age at diagnosis in this survey was 4.3 years, similar to the mean age at diagnosis of 4.43 years reported by the United States-based Parent Project Muscular Dystrophy [7] and much younger than the mean age at diagnosis of 7 years in Thailand [8]. The percentage of ≥5-year-old patients using corticosteroids was 71.3%, which exceeds the figure of 46.4% reported for ≥5-year-old patients in the United States [9] and the figure of 49.7% reported in the Translational Research in Europe for the Assessment and Treatment for Neuromuscular Disorders (TREAT-NMD) DMD database [10].

Given the increased attention that rare diseases have recently received in China, DMD has become more familiar to the general public and physicians in China. For our part, we have launched the Chinese national DMD registry, opened a multidisciplinary outpatient clinic, promoted routine follow-up appointments, and developed the certified "One City, One Doctor" program to implement standardized care at other neuromuscular centers around the country [11]. The participation of Chinese sites in the ongoing global clinical trials of ataluren and viltolarsen has also promoted the implementation of standardized care. However, some challenges remain. Cultural and economic barriers have prevented parents from accepting the use of a ventilator for DMD patients. Patients in China have a poor adherence to respiratory management recommendations for DMD [12]. Therefore, we held regular patient education to operationalize the standard care considerations in neuromuscular centers around the country.

The question of whether patients with DMD should maintain a certain amount of voluntary exercise has long been subjected to debate. Kogelman et al. [13] found that voluntary exercise improved heart and skeletal muscle function in a mouse model of DMD. Human studies have shown that lower physical activity levels are associated with muscle weakness in adults with DMD [14], and a study of dystrophic worms showed that longevity was negatively associated with physical exertion regardless of effort duration [15]. Interestingly, in our

study, 76 and 57 patients lost independent ambulation in September and March, respectively. This suggests that there may be "hot months" in which there is a loss of independent ambulation ability, a finding that has not been reported previously. July and August are the summer vacation months for students in China, so their daily activity levels are dramatically reduced as they watch TV, play online games, and immerse themselves in social media instead of going out to school [16]. January and February are the coldest months in China, and wearing heavy clothes and taking winter vacation time may also limit students' daily activity levels. Given that sedentary behaviors can cause deterioration of motor functions, we speculate that the phenomenon of "hot months" could be related to the patients' reduced daily activity levels in the preceding months. Notably, September and March did not stand out for the 154 participants who lost independent ambulation ability in 2020. Our results raise the question of whether physical activity and daily activities help maintain motor functions in patients with DMD. For patients with DMD, intense activities could lead to excessive damage to muscle membranes, but sedentary behaviors may lead to muscular atrophy and muscle weakness. Further research is therefore needed to determine the optimal levels of activity, and we have designed further multicenter studies to verify our results.

The COVID-19 pandemic has, of course, created a major additional challenge for the implementation of standardized care. Patients with chronic diseases such as DMD are generally at greater risk of severe illness or death if they contract COVID-19, and the need for social distancing means that patients face considerable challenges in accessing clinical services. Our survey results showed that patients with DMD are experiencing reductions in outside activities and visits to rehabilitation services and accelerated declines in motor function. Accelerated motor decline refers to a sharp deterioration of motor function based on the feelings or observations of each patient and/or their guardians. We noted in the questionnaire that the patients or their guardians observed an obvious decline in motor function during the pandemic compared to the normal disease progression before the pandemic. Our results also pointed out that they expected more assistance from the society than they had received before the pandemic.

Our findings mirror those reported internationally. Studies in Europe have found that 40% of surveyed neuromuscular centers report reductions in outpatient visits, and rehabilitative services were the most affected [4,17]. An Italian study of patients with neuromuscular disorders confirmed that COVID-19 related quarantines had reduced patient engagement in physical activity, especially walking activities [18]. In Japan, 30% of patients with muscular dystrophy postponed regular consultations, and 47.2% reported decreased physical activity [19]. One difference from overseas results was that our data showed that 60.8% of patients expected more assistance from society than they had received before the pandemic whereas a study in Japan found that only 19.3% of patients with muscular dystrophy had similar expectations [19].

To mitigate the pandemic's adverse effects on patients with DMD, clinicians and public officials should work to improve healthcare accessibility. Patients may benefit from the provision of more at-home services for maintaining sufficient daily activity, personal and in-home rehabilitation support, and nutritional and weight management services.

Telemedicine and wearable smart devices played important roles in neuromuscular care during the pandemic, with telemedicine providing a convenient medium for clinician-patient interaction that improved patients' access to medicine, reduced their travel costs, and avoided infection risks [20,21]. Given these benefits, telemedicine systems have been established and applied to neuromuscular care around the world [22], with different countries having different acceptance rates for telemedicine. An evaluation of telemedicine in the United Kingdom showed that it was feasible for patients with motor neuron disease [23]. In Europe, most patients reported that remote consultations were either "just as good" as face-to-face consultations (67.1%) or even "better" than face-to-face consultations (9.0%) [24]. In contrast, a survey in Ireland showed that only 13% of new patients with neuromuscular disorders were satisfied with remote consultations, with the study's authors suggesting that teleconsultations were more suitable for patients with headache and epilepsy than for those with neuromuscular disorders [25]. Issues such as a lack of physical examination and restricted time for effective teleconsultation were the most reported reasons for patient dissatisfaction with telemedicine, when compared to outpatient consultations.

Our data showed that most patients thought telemedicine could partially solve the problem of poor access to medical services for patients during the pandemic, with the percentages of patients with favorable impressions being 71% in non-lockdown areas and 61.2% in lockdown areas. Access to the Internet via smartphones makes telemedicine a feasible option for patients with chronic conditions [26]. Furthermore, we supposed that convenient electronic payment options and faster transportation logistics (for essential medicine access) in China contribute to satisfactory telemedicine experiences. The comprehensive development of telemedicine is advisable for providing a better user experience, with studies confirming that training and predesigned formats can improve the quality of these consultations [27].

During the pandemic, telemedicine in China may partially solve the problem of access to essential medicines, but the price is also an important factor in drug avail-

ability. Several new disease-modifying drugs for neuromuscular diseases have received approval from the FDA/EMA in recent years, and this brings hope for effective treatment. The ease of online access to the Internet makes it convenient for patients and guardians to get more information about the new disease-modifying drugs. Nearly ninety percent of the respondents would like to take these drugs if they are eligible for the drugs. However, most of these drugs are not accessible to patients due to their high prices. For example, onasemnogene abeparvovec, a gene therapy drug for spinal muscular atrophy (SMA), is regarded as the most expensive gene therapy drug, with an expected annual price of approximately €2,000,000. Few families can afford this price by themselves, even in developed countries [28]. Enzyme replacement therapy with Alglucosidase alfa is also not cost-effective for the treatment of adult Pompe disease [29]. For patients with DMD, exon-skipping and stop-codon readthrough drugs have secured FDA and EMA approval, but only 0.6% of our survey respondents' parents could afford these drugs at the prices charged in Europe and the United States.

Although these prices create a serious accessibility challenge for patients with DMD, especially in developing countries, nusinersen, a drug for SMA that received approval in China in 2019, sets a good precedent for DMD drugs. In some Chinese cities, 86.4% of the price of nusinersen is covered by medical and commercial insurance. Nusinersen and some other drugs for treating rare diseases will be added to China's national insurance drug list from the beginning of the coming year (2022). Collaboration between governmental and non-governmental entities, such as charities, insurance companies, corporations, and individuals, is required to tackle this problem.

This study is a large survey of the health care status of DMD patients in China during the COVID-19 pandemic, but all participants were from our national registry database or our WeChat groups, which may introduce a sampling bias. Another limitation of this study was that this study is based on a questionnaire survey of patients and/or their families; therefore, motor function was not evaluated properly. Additionally, different virus control measures applied in different areas may have led to a heterogeneous impact on patients' access to health care.

To conclude, the implementation of standardized care for DMD in China is consistent with global practices, and the COVID-19 pandemic has affected the care of patients with chronic diseases worldwide. Our data show that many patients with DMD reduced their outside activities, avoided rehabilitation services, postponed follow-up appointments, and experienced motor function declines during the pandemic. These complaints were more frequent among those patients who lived in areas subjected to lockdowns. Telemedicine may be an effective model for providing healthcare to patients during a pandemic.The lack of affordability of new disease-modifying drugs is a challenge in China and other developing countries, and collaboration between governmental and non-governmental entities is necessary to make these new drugs accessible.

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Conflict of Interest Disclosures

The authors declare that they have no competing interests.

Availability of data and materials

The datasets used during the current study are available from the corresponding author on reasonable request.

Disclosures

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Author contributions

(I) Conception and design: SZ, SWu, YT.
(II) Administrative support: SWu,YD, SWang, CZ.
(III) Provision of study materials or patients: SZ, SWang, MH, SK.
(IV) Collection and assembly of data: SZ, YD, SWang, XC, MH, SK.
(V) Data analysis and interpretation: SZ, XC, MH, SWu, YT.
(VI) Manuscript writing and review: All authors.
(VII) Final approval of manuscript: All authors.
The authors are accountable for all aspects of the work and for ensuring that questions related to the accu-

racy or integrity of any part of the work are appropriately investigated and resolved.

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