Influence of A Public Health Emergency On Care For Patients With Duchenne Muscular Dystrophy: An Online, Cross-Sectional Survey

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Abstract

Background: The public health emergency has created challenges for the care of patients, particularly those with chronic diseases such as Duchenne muscular dystrophy. To elucidate the challenges faced by Chinese patients with Duchenne muscular dystrophy during the public health emergency coronavirus disease 2019 pandemic, we conducted an online cross-sectional survey, the responses of which were collected between March 27 and June 30, 2021.

Results: In total, valid questionnaire responses were obtained from 2,105 patients, of whom 49 lived in pandemic lockdown areas. Of the 2,056 responders from non-lockdown areas, 42.8% reduced their outside daily activities, 49.4% reduced their use of rehabilitation services, 39.7% postponed regular follow-up appointments, and 40.8% complained of accelerated declines in motor function over the previous year. The corresponding figures for the 49 participants from lockdown areas were almost all higher, with 67.3% reducing outside daily activities, 44.9% reducing their use of rehabilitation services, 79.6% postponing regular follow-up appointments, and 55.1% complaining of accelerated declines in motor function. When asked whether they expected more assistance from society than they had received before the pandemic, 60.8% of patients in non-lockdown areas and 87.8% of those in lockdown areas responded affirmatively. When asked whether they felt more anxious than they had before the pandemic and needed psychological counseling, 11.5% of respondents in non-lockdown areas and 18.4% of respondents in lockdown areas responded affirmatively. In non-lockdown areas, 76% of respondents had at least one telemedicine visit, and 71% of them thought that telemedicine was helpful. In lockdown areas, 91.8% had used telemedicine at least once, and 66.7% of them found it helpful.

Conclusions: These public health emergency control measures have affected the care of patients with chronic diseases worldwide, particularly pronounced in lockdown areas. It is imperative that healthcare workers assist patients and establish more robust chronic disease management systems. Telemedicine is an effective model for providing healthcare to such patients.

Background

Duchenne muscular dystrophy (DMD) is an X-linked neuromuscular disorder 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 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 immunosuppression and increased vulnerability to infections. Meanwhile, respiratory muscle weakness in patients with DMD can cause a weak cough, atelectasis, pneumonia, and respiratory failure.

Since late 2019, the coronavirus disease 2019 (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 (2). To prevent COVID-19 infections, many patients with DMD reduced their hospital follow-up visits and usage of rehabilitation services. This exemplifies how emergency responses to the COVID-19 pandemic have created unprecedented difficulties in the management of chronic diseases.

To better understand the challenges faced by patients with DMD in China during the pandemic, we conducted a cross-sectional survey. There is little research of this type available for DMD patients in China.

Results

Respondent profiles

We obtained valid questionnaire responses from 2,105 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 1,607 participants who were ≥5 years old and lived in non-lockdown areas, 1,234 (70.8%) received corticosteroid treatment, 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 2,105 respondents, 78.1% (n=1,646) remained independently ambulatory. Of the 459 non-ambulatory patients, 10.0% (n=46) used part-time nocturnal noninvasive ventilation, and the remaining 90.0% (n=392) never used assisted ventilation. The months in which the majority of patients experienced 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 (Figure 1). In the 305 patients who experienced loss of independent ambulation before 2020, this loss of ability was also 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 (Table 1).

Table 1
Number of patients with DMD who experienced loss of ambulation during each month.

No. of LOA Month	Jan	Feb	Mar	Apr	May	Jun	Jul	Aug	Sep	Oct	Nov	Dec
Total	36	24	76	20	35	28	29	32	57	45	42	35
Before 2020	16	17	47	10	24	22	20	21	41	33	28	26
2020	11	5	11	10	10	6	9	11	16	12	14	9

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

Of the responders from non-lockdown areas, 42.8% (n=880) reduced their outside daily activities, 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 (Figure 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 2.

Table 2

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	<i>P</i> 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 than before	60.8% (1250/2056)	87.8%(43/49)	<0.001(***)
Need of psychological counseling	11.5%(236/2056)	18.4%(9/49)	0.137
(* <i>p</i> <0.05; ** <i>p</i> <0.01; *** <i>p</i> <0.001.)			

DMD: Duchenne muscular dystrophy

Of the responders from non-lockdown areas, 60.8% (n=1,250) expected to receive more assistance from 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.

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

Of the respondents from non-lockdown areas, 76% (n=1,563) had at least one telemedicine visit, and 71% (n=1,109) of those who utilized telemedicine thought that it was helpful. 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.

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=1,826) 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=1,871) of our respondents would like to participate in global clinical trials assessing new drugs for DMD.

Discussion

Standardized management strategies are crucial for patients with neuromuscular diseases such as DMD, with studies showing that implementing standardized management of DMD delays the loss of ambulation and extends overall survival (3, 4). As evidenced by the publication of national clinical care guidelines for DMD, standardized management for patients DMD has been promoted and implemented in China. Our own survey results testify to the benefits of such standardized management, with all respondents having visited a specialized neuromuscular center for at least one visit. The mean age at diagnosis in this survey was 4.3 years, which is similar to the mean age at diagnosis of 4.43 years reported by the US-based Parent Project Muscular Dystrophy (5) and much younger than the mean age at diagnosis of 7 years in Thailand (6). 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 (7) and the figure of 49.7% reported in the global TREAT-NMD DMD database (8).

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 (9). 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. For example, regarding accepting the use of

ventilators, patients in China had poor adherence to respiratory management recommendations for DMD (10).

The question of whether patients with DMD should maintain a certain amount of voluntary exercise has long been subject to debate. Kogelman et al. (11) 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 (12), and a study of dystrophic worms showed that longevity was negatively associated with physical exertion regardless of effort duration (13). Interestingly, in our study, 76 and 57 patients lost independent ambulation in September and March, respectively. This suggests that there may be “hot months” that trigger the 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 students’ 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 (14). 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. In the first half of 2020, primary and secondary school students in China took online classes at home, thus sparing them the need to perform certain otherwise-essential daily activities during school months. 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 activity 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 own survey results showed that patients with DMD are experiencing reductions in outside activities and visits to rehabilitation services and accelerated declines in motor function. Our results also pointed out that they expected more assistance from society than they had received before the pandemic. Overall, our survey data point to three key findings. First, the measures taken to reduce virus transmission, such as traffic restrictions, lockdowns, and community quarantines, unavoidably influence patients’ access to daily activities outside their homes. Second, the particular vulnerability of patients with DMD caused patients to take steps to reduce their risk of contracting COVID-19, such as reductions in their usage of hospital follow-up appointments and rehabilitation services. Third, reductions in physical activity, the avoidance of rehabilitation services, and accelerated declines in motor function were reported by our participants during 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 (15, 16). An Italian study of patients with neuromuscular disorders confirmed that COVID-19 related quarantines have reduced patient engagement in physical activity, especially walking activities (17). In Japan, 30% of patients with muscular dystrophy postponed regular consultations, and 47.2% of patients reported decreased physical activity (18). Even today, when control actions have effectively addressed the pandemic in China, migration controls are still encouraged to manage sporadic urban outbreaks, especially in lockdown areas. 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 (18).

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 (19, 20). Given these benefits, telemedicine systems have been established and applied to neuromuscular care around the world (21), with different countries having different acceptance rates for telemedicine. An evaluation of telemedicine in the UK showed that it was feasible for patients with motor neuron disease (22). 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%) (23). The authors of that study suspected that the positive feedback was likely influenced by the patients' feelings of apprehension around physically attending a hospital during the pandemic. 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 better suited for patients with headache and epilepsy than for those with neuromuscular disorders (24). Issues such as a lack of physical examination and restricted time for effective teleconsultation were proposed as reasons for patient dissatisfaction with telemedicine.

Our data showed that most patients thought that telemedicine was helpful, 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 (25). Furthermore, 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 (26).

During the pandemic, telemedicine in China may partially solve the problem of access to essential medicines, but price is also an important factor in drug availability. Several new disease-modifying drugs for neuromuscular diseases have received approval in recent years, and this brings hope for effective treatment. 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 (27). Enzyme replacement therapy with alglucosidase alfa is also not cost-effective for the treatment of adult Pompe disease (28). 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.

With the advancement of gene therapy techniques, the approval of additional candidate drugs can also be expected over the coming years, and gene addition drugs could be even more expensive than PTC-124 (ataluren) or exon-skipping drugs. These prices create a serious accessibility challenge for patients with DMD, especially in developing countries, but the case of 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 73 other drugs for treating rare diseases will be added to China's national insurance drug list from the start of next year (2022). Other potential strategies for addressing affordability include mandating lower prices, optimizing insurance reimbursement, and reducing development costs. Collaboration between governmental and nongovernmental 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. Limitations of the study also included that specific reasons for not using a ventilator were not further investigated. Additionally, different virus control measures applied in different areas may have led to a heterogeneous impact on patients' access to health care.

Conclusions

The implementation of standardized care for DMD in China is consistent with practices abroad, 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 accelerated motor function declines during the pandemic. These complaints were more frequent among those patients who lived in areas subjected to lockdowns. Furthermore, many patients accessed telemedicine during the pandemic and reported that it was helpful. It is imperative that healthcare services assist these patients and establish more robust chronic disease management systems. Telemedicine is an effective model for providing healthcare to patients during a pandemic. The lack of affordability of new disease-modifying drugs is a challenge not only in China but

also in other developing countries, and collaboration between governmental and nongovernmental entities is necessary to make these new drugs accessible.

Methods

Questionnaire design

We conducted a cross-sectional survey of patients with DMD through a Chinese questionnaire site (www.wjx.cn) and WeChat (social media platform). 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 was concerned with patients' access to medical care, including both 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. 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. The questionnaire was available between March 27 and June 30, 2021.

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 $p < 0.05$.

List Of Abbreviations

Duchenne muscular dystrophy (DMD)

coronavirus disease 2019 (COVID-19)

US Food and Drug Administration (FDA)

European Medicines Agency (EMA)

spinal muscular atrophy (SMA)

Declarations

Ethics approval and consent to participate

This study was approved by our ethics committee (No.KY2121-001). Online informed consent was obtained from patients or their legal guardians.

Consent for publication

Consent for publication was obtained from the patients and/or their parents or legal guardians.

Availability of data and materials

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

Competing interests

The authors declare that they have no competing interests.

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Authors' 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: All authors
- (VII) Final approval of manuscript: All authors

The authors are accountable for all aspects of the work in ensuring that questions related to the accuracy or integrity of any part of the work are appropriately investigated and resolved.

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Figures

Figure 1

Number of patients with DMD who experienced LOA during each month.

LOA: loss of ambulation, DMD: Duchenne muscular dystrophy

Figure 2

Main influence of public health emergency control measures on healthcare for patients with DMD.

DMD: Duchenne muscular dystrophy