3477, 98.86%

3250, 92.41%

3276, 93.15%

2794, 79.44%

Analysis of Health-Related Quality of Life and Its Influencing Factors in



Chinese Patients With Duchenne Muscular Dystrophy

Shanshan Wang^{1†}, Yun Yuan², Anqi Wang¹, Yunshu Xu¹, Zhiying Xie², Kun Wu³, Ni Yuan^{1*}

1 School of Public Health, Dalian Medical University, Dalian, China.

- 2 Peking University First Hospital, Beijing, China.
- 3 Pansy Rare Disease Alliance, Shanghai, China.

INTRODUCTION

- Duchenne muscular dystrophy (DMD) is the most prevalent X-linked recessive genetic muscle degenerative disease, and was listed in the First Catalogue of Rare Disease List issued by China National Health Commission in 2018.
- Currently, there is no curative therapy for DMD.

 Patients experience significantly lower healthrelated quality of life (HRQoL) both physically
 and psychologically compared to the general
 population, and their HRQoL deteriorates as the
 disease progresses.

OBJECTIVE

This study aimed to evaluate HRQoL and its influencing factors among Chinese patients with DMD.

METHODS



Dec. 2024 – Jan. 2025



Clinicians & patient organizations



Online-Survey



3,517 Participants



Descriptive Statistics



Multivariate Linear Regression

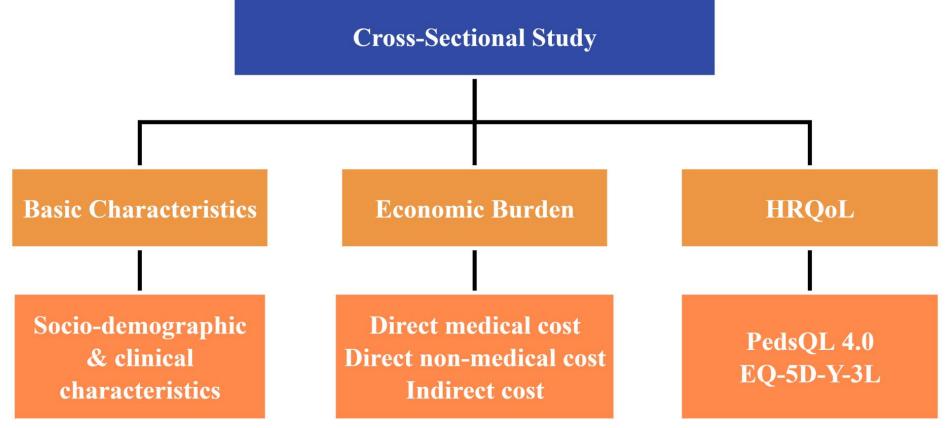


Fig.1 A Multi-dimensional Assessment Framework for DMD: A Cross-sectional Study Flowchart

REFERENCE

Chronic pain, psychological distress, and quality of life in males with Duchenne muscular dystrophy.

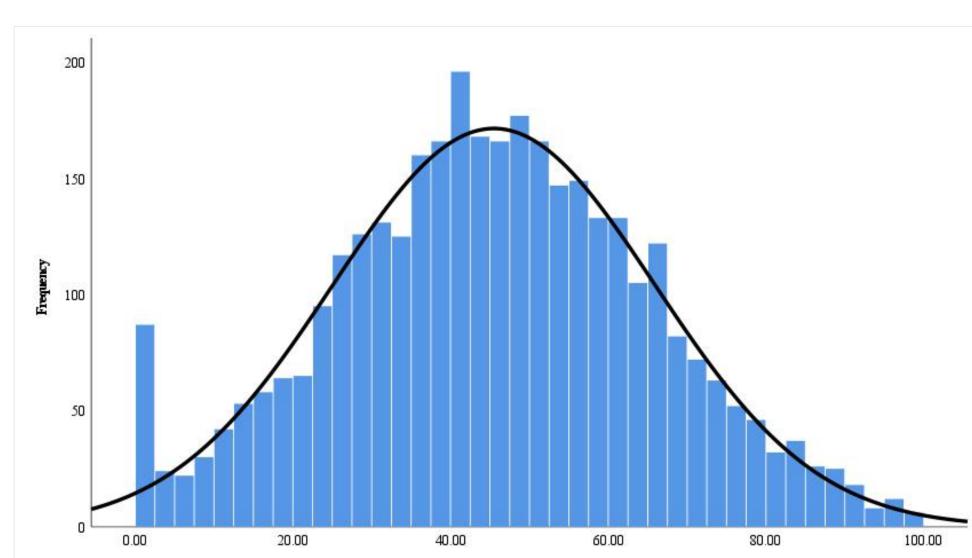
RESULTS

1. Basic Characteristics

- The patients' mean (SD) age was 8.95 (3.27) years; 98.86% were male, as shown in Fig.2.
- Most patients (79.44%) were currently enrolled in school.
- The coverage rate of Basic Medical Insurance was 93.15%.

2. Economic Burden

• 87.35% of the patients had outpatient or inpatient visits in the past year, as shown in Fig.3.



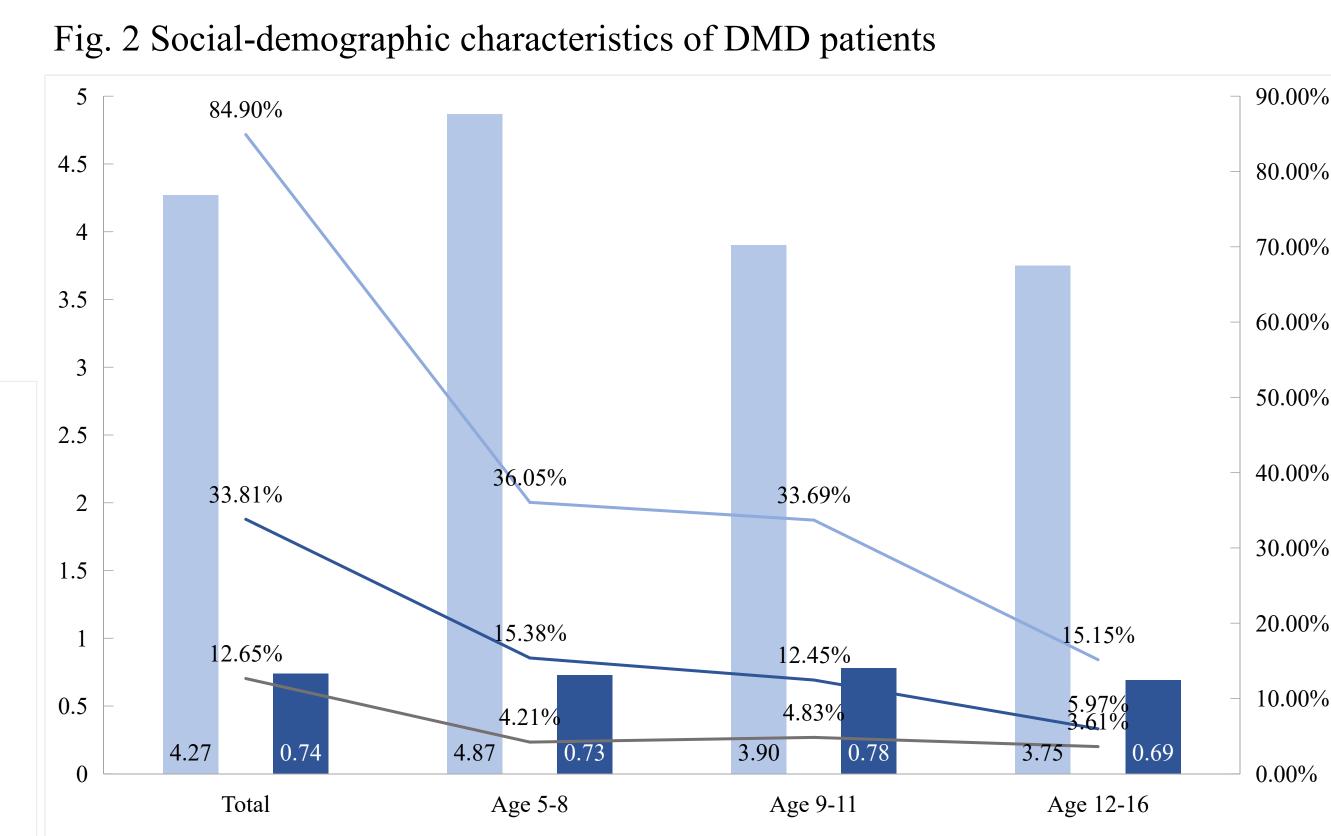
PedsQL score

Fig. 4 Frequency distribution of PedsQL scores in DMD patients

— Proportion of outpatients

Fig. 3 Annual healthcare resource utilization of DMD patients

— Neither outpatient nor hospitalisation visits



Female 40, 1.14%

Never go to school 169, 4.81%

Graduated | 24, 0.68%

Be going to school

Supplementary medical insurance 212, 6.03%

Commercial health insurance 292, 8.30%

Basic Medical Insurance

Drop out of school and stay at home 530, 15.07%

Others 267, 7.59%

1453, 41.31%

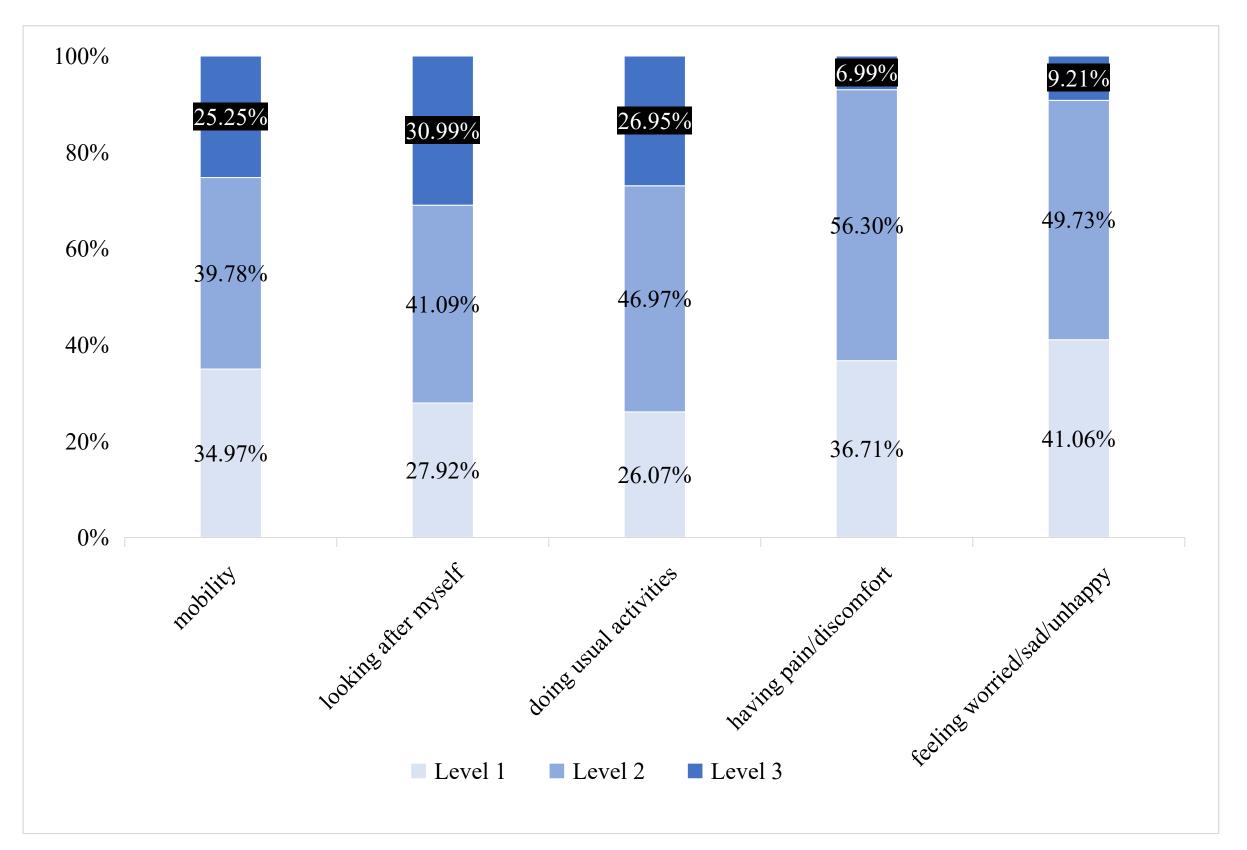


Fig. 5 Sample distribution of EQ-5D-Y-3L of DMD patients

3. HRQoL

Number of outpatient visits

• The mean (SD) PedsQL 4.0 score was 45.50 (20.46), with substantial challenges in *physical functioning*, as shown in Fig.4.

Number of hospitalisation visits

— Proportion of hospitalized

• The mean (SD) EQ-VAS score was 66.52 (22.58), and the mean (SD) health utility score was 0.61 (0.29).

Patients experienced significant challenges in *looking*after myself (72.1%) and doing usual activities (73.9%), as shown in Fig.5.

4. Influencing Factors

• Age and current clinical characteristics (high serum creatine kinase, difficulty sitting up in the supine position, dysgaitosis, achilles tendon contracture, and scoliosis) are significant factors influencing EQ-5D-Y-3L scores and PedsQL 4.0 scores.

CONCLUSION

• This study demonstrated that Chinese patients with DMD have markedly reduced HRQoL, particularly in the physical domain. These findings provide empirical evidence to inform interventions aimed at improving HRQoL for DMD patients.