



Analysis of Health-Related Quality of Life and Its Influencing Factors in Chinese Patients With Duchenne Muscular Dystrophy

RWD18

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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 health-related 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

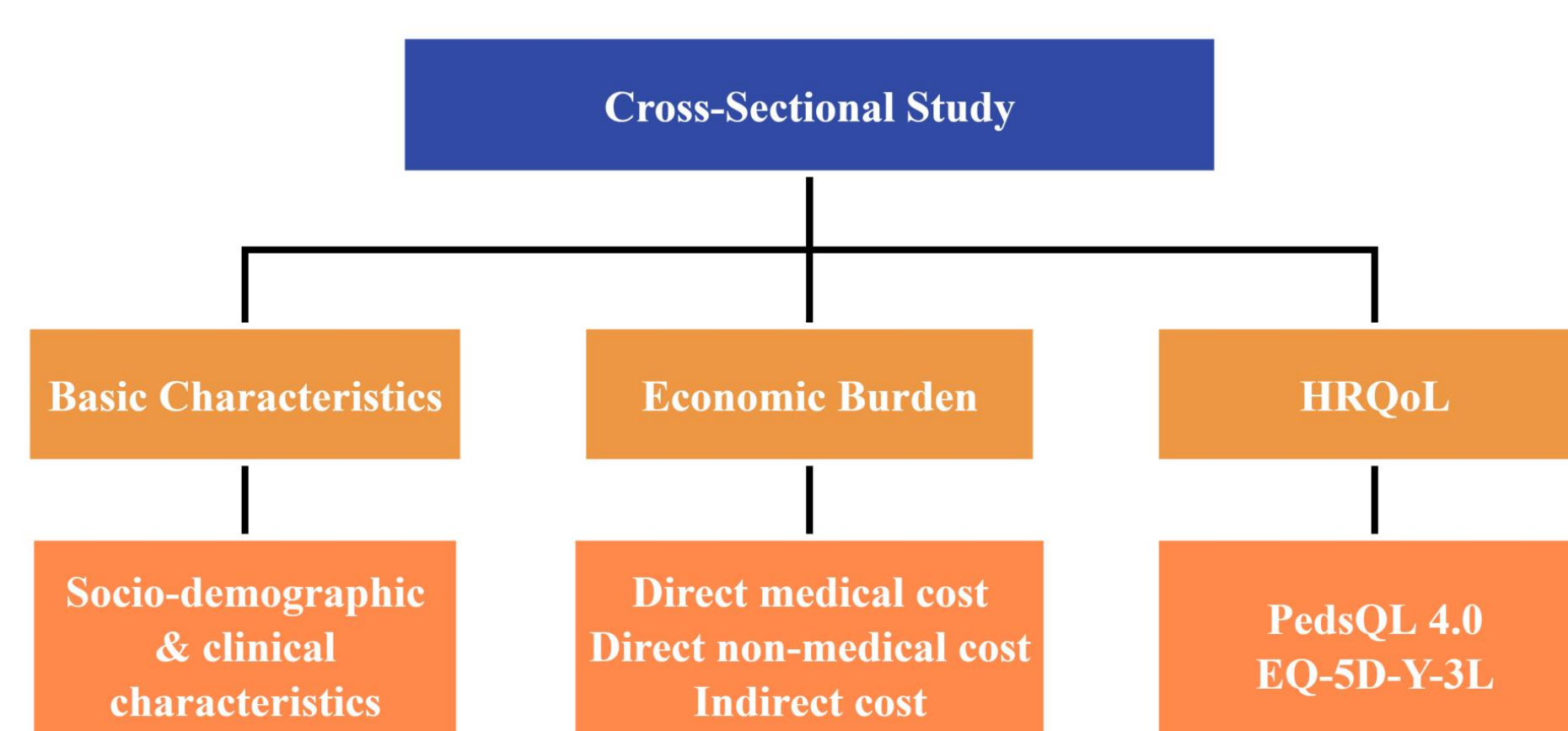


Fig.1 A Multi-dimensional Assessment Framework for DMD: A Cross-sectional Study Flowchart

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.

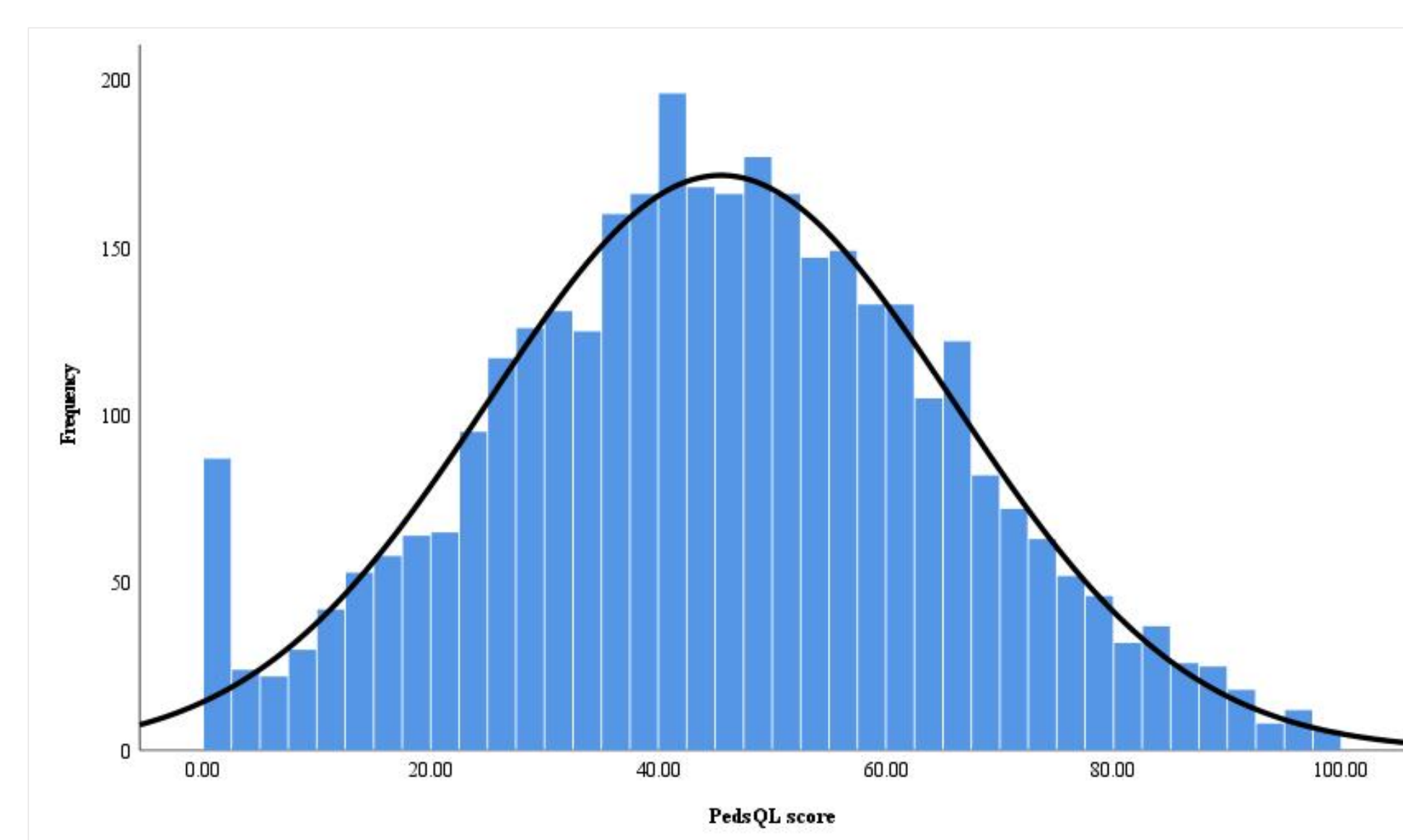


Fig. 4 Frequency distribution of PedsQL scores in DMD patients

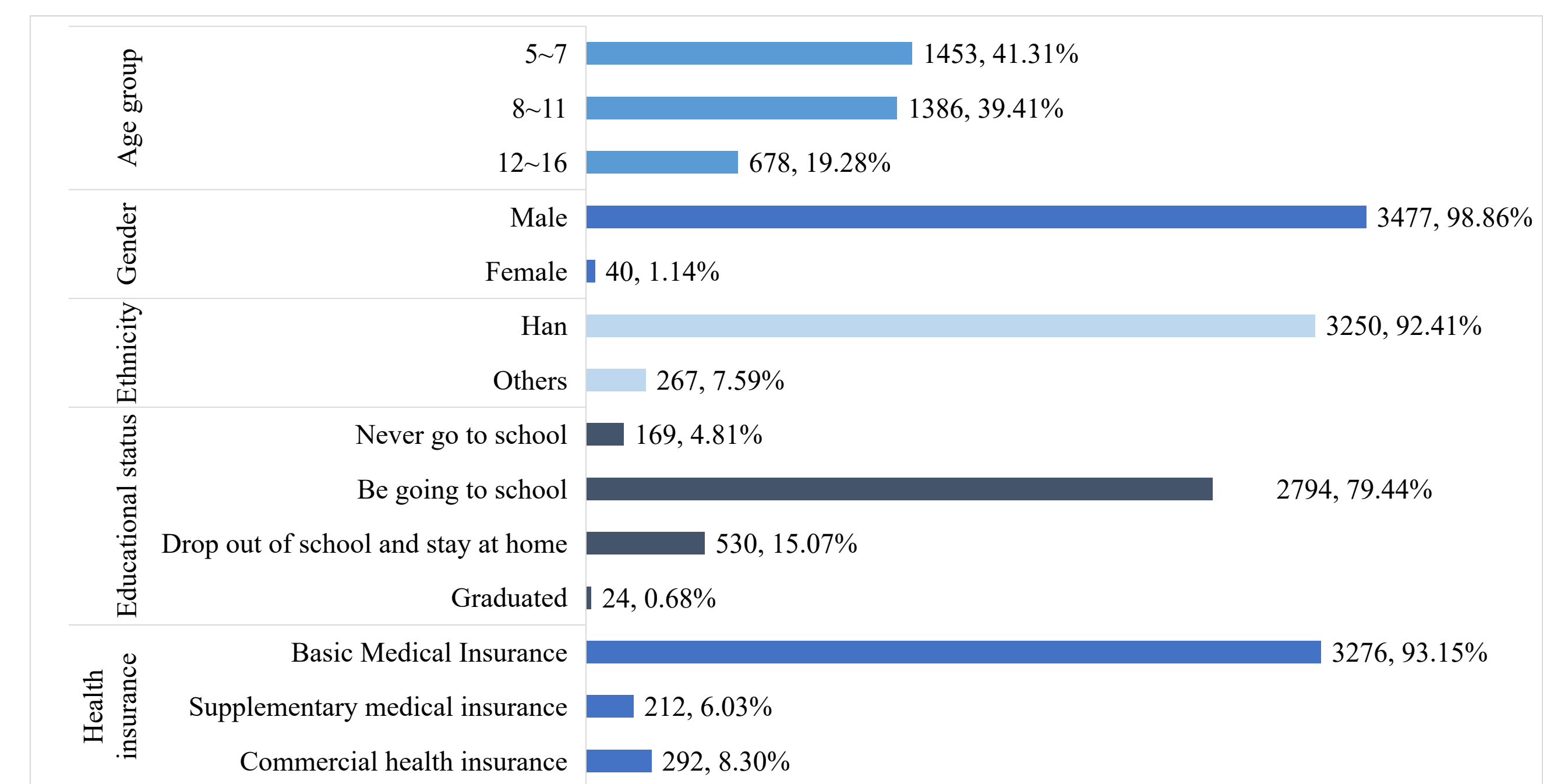


Fig. 2 Social-demographic characteristics of DMD patients

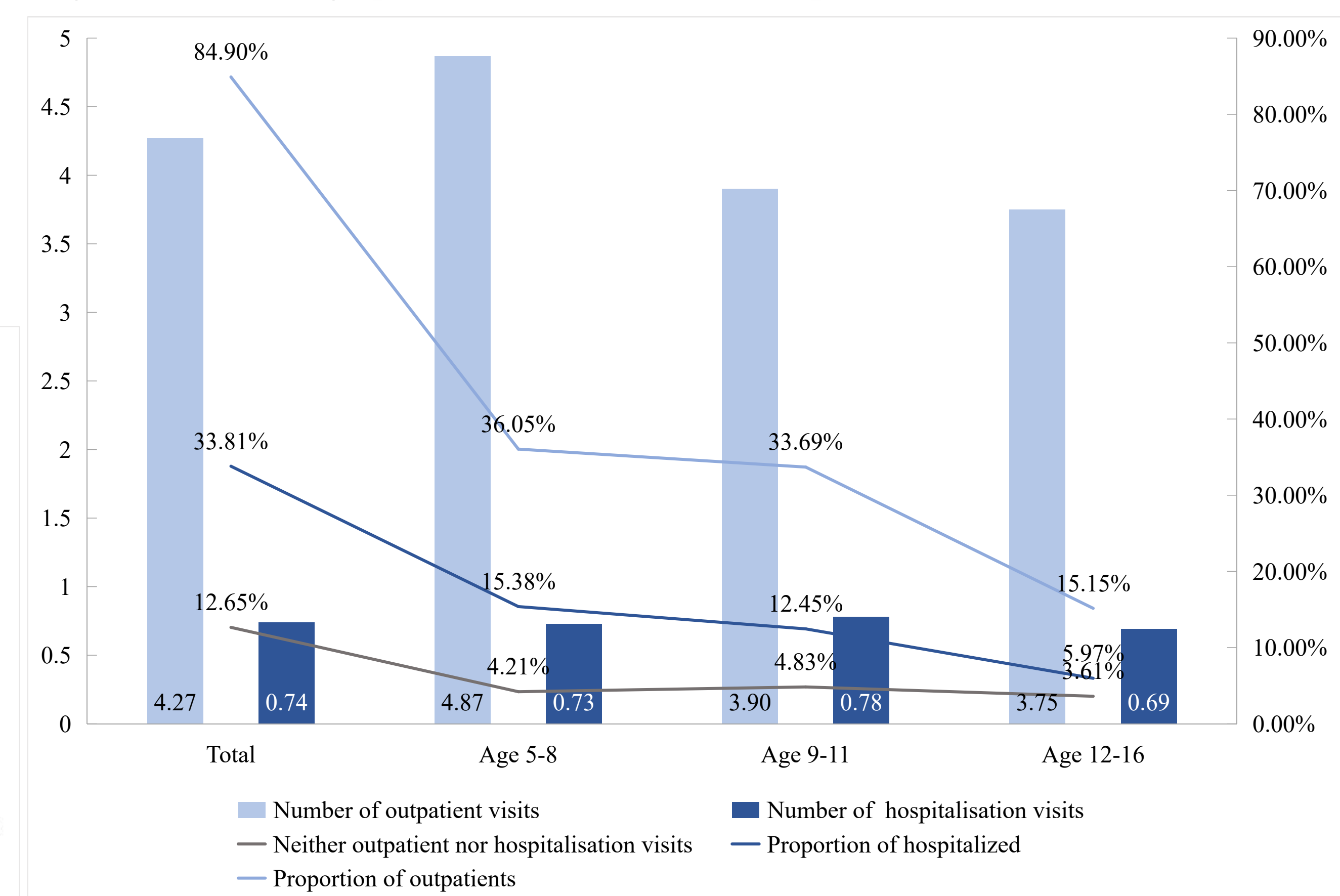


Fig. 3 Annual healthcare resource utilization of DMD patients

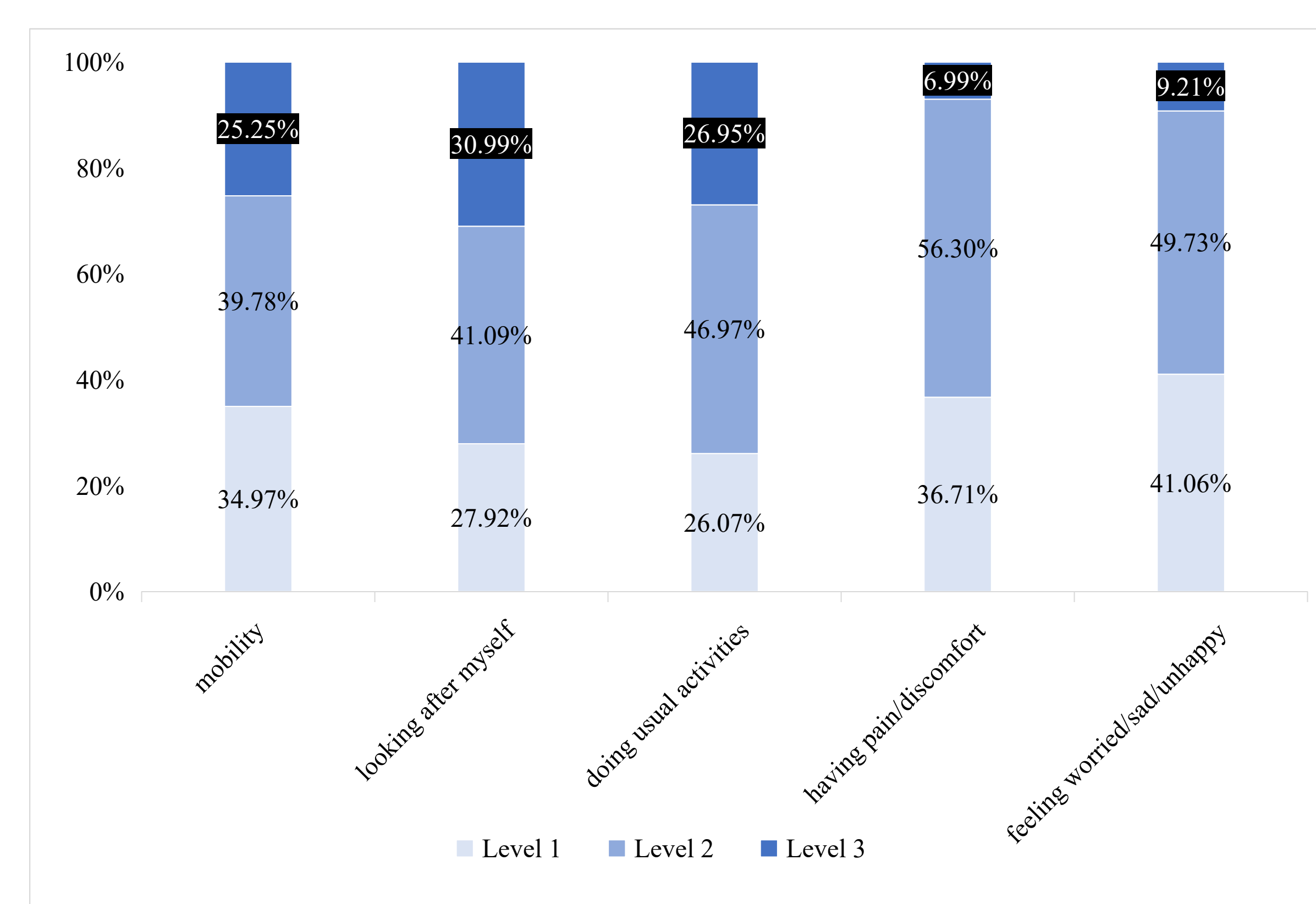


Fig. 5 Sample distribution of EQ-5D-Y-3L of DMD patients

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.