LIVING WITH DUCHENNE MUSCULAR DYSTROPHY: MEDICAL, PSYCHOSOCIAL AND FINANCIAL BURDEN

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Objectives

Duchenne muscular dystrophy (DMD) is a rare neuromuscular disease affecting approximately 300 people in the Czech Republic (CZ). In the literature, several studies have been performed examining demographic characteristics, medical conditions and quality of life of the patients[1,2,3,4]. However, to our knowledge, there is a lack of evidence describing patients' and their caregivers' work productivity and the financial burden of the disease.

Therefore, our study aimed to explore medical, psychosocial and financial aspects of DMD in CZ and to describe how the disease influences the quality of life, family budget and work productivity of patients and their caregivers.

Methods

The survey was divided into four sections:

1) state of the disease and available care,
2) quality of life measured by EQ-5D (using visual analogue scale (VAS) and Utility Index) and PedsQL questionnaires,
3) financial burden from patients' perspective (i.e. only out-of-pocket payments including co-payments for medical devices and medication, health insurance costs were not described),
4) work productivity (Work Productivity and Activity Impairment Questionnaire (WPAI)).

The results were collected from 63 patients covering approximately 20% of patients in CZ.

Patients were approached by their neurologists and by patient organizations Parent Project, z.s. and End Duchenne. Data were collected from January 2018 to April 2019.

Results

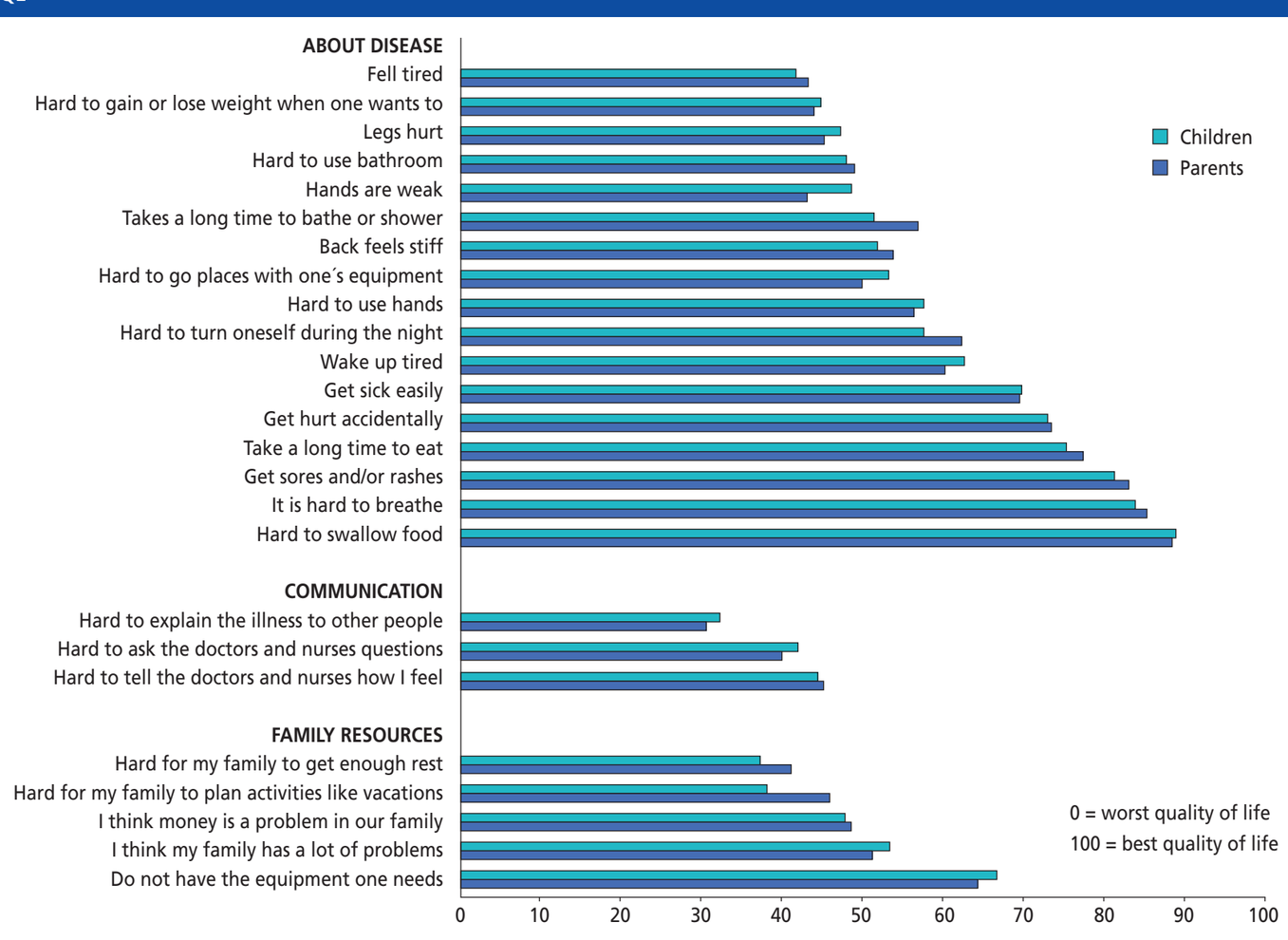
Demographic characteristics of the patients are shown in **Table 1**. The median age of respondents was 12.9 years with median age at diagnosis of 3.0 years and loss of ambulation at 9.5 years. Sixty-one respondents (96.8%) were at least annually examined by a neuromuscular specialist, while 80.6% of these patients were satisfied with the care provided and 69.8% felt they had been offered sufficient help at diagnosis. Sixty-five percent of respondents are part of a patient organization.

Corticosteroids were never used by 28.6% of patients. However, 23.8% of respondents were less than 8 years old. Among non-ambulant patients, 10 out of 29 were still on corticoids. Nineteen patients were hospitalized at least once in the last year, with 85.2% of planned hospitalizations.

Table 1. Patient characteristics

	n	%
Under 5 years	5	7.9%
5–7 years	10	15.9%
8–12 years	23	36.5%
13–18 years	14	22.2%
Above 18 years	11	17.5%
	n	mean, median (±SD)
Age	63	13.7, 12.9 (±5.7)
Age at diagnosis	62	3.4, 3.0 (±2.5)
Loss of ambulation	28	9.4, 9.5 (±1.9)
	n	%
Neuromuscular specialist visit annually	61	96.8%
Sufficient help at diagnosis	44	69.8%
Part of patient organization	41	65.1%
Satisfied with care provided	50	80.6%

Figure 2. PedsQL



Patients were seen by a physiotherapist on average once every two weeks while 85.7% of respondents exercised at least a few days a week. Out of 19 respondents who have completed their education, four finished high school and one had a college degree.

Fifty respondents (79.4%) stated that the diagnosis decreases their quality of life. The respondents' self-rated health status reported by visual analogue scale was on average 82.5 in children up to 5 years of age, then decreased with age until it reached 47.5 in respondents 18+ years old. The EQ-5D Utility Index markedly decreased from 0.712 in children under 5 years to 0.026 in 18+years group (**Figure 1**). We would like to stress that adult patients have EQ-5D index almost equal to 0 which indicates lower bound, i.e. death state; the quality of life of adult patients is thus severely impaired.

In PedsQL, the worst-rated ability of DMD patients by both respondents and their parents was communication about the disease with professionals as well as non-professionals (**Figure 2**). Patients and their families also reported difficulties in organizing events such as family holidays or rest in general. Other most frequently reported complications were fatigue, leg pain and weight control. Highest rated abilities (i.e. the abilities imposing the least amount of difficulties) were swallowing and breathing.

Regarding financial burden of DMD, caregivers stated that on average 20% of family's income is spent on the care of DMD patient, monthly €54 on vitamins or other dietary supplements, €42 on medical devices, €41 on travel costs, €21 on medication and €39 on others (e.g. specialized exercise, shoes, insoles) (**Figure 3**).

Family caregiver dedicates on average 16.3 hours/day to taking care of a DMD patient. Moreover, 64.5% caregivers are not employed most likely due to DMD caregiving. The average deterioration in labor productivity of employed carers is 37.7% (work impairment WPAI); usual activities are impaired by 47.8% in all caregivers (activity impairment WPAI) (**Figure 4**).

Conclusions

The study provides an overview and insight into the overall burden of DMD patients and their caregivers in CZ and addresses several important issues. Not only does DMD have a tremendous effect on the quality of life of the patient, but it also has a pronounced impact on caregivers' daily activities and significantly impairs their ability to work. This loss of productivity further deepens the financial burden imposed by a family member diagnosed with DMD. To our knowledge, this is the first DMD patient and caregiver study of this kind conducted in the Czech Republic and one of the few worldwide[5].

Acknowledgment: *We thank Bioxa Therapeutics for Parent Project support inducing this project.*

Figure 1. Quality of life

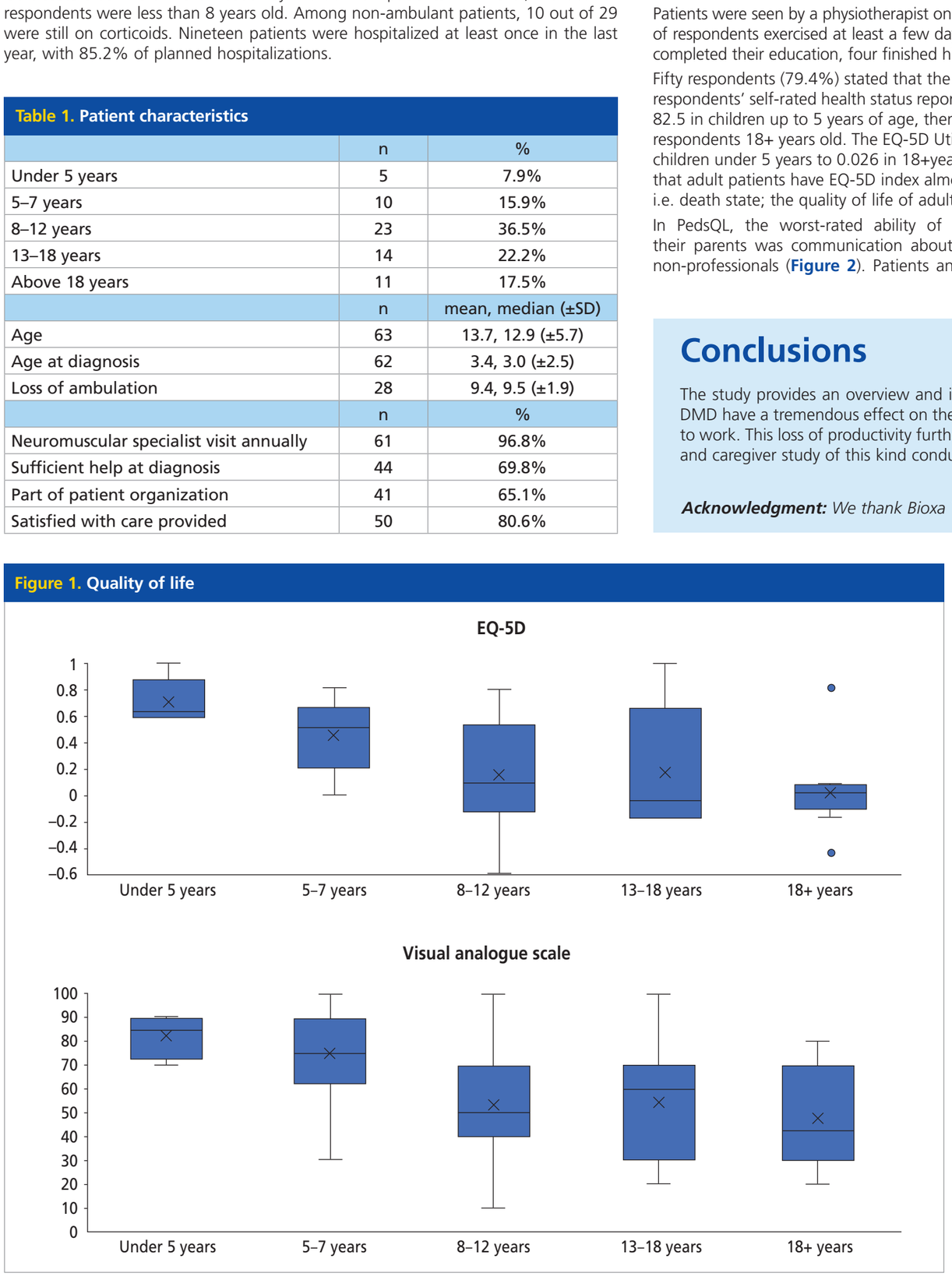


Figure 3. Family's income and average costs per month

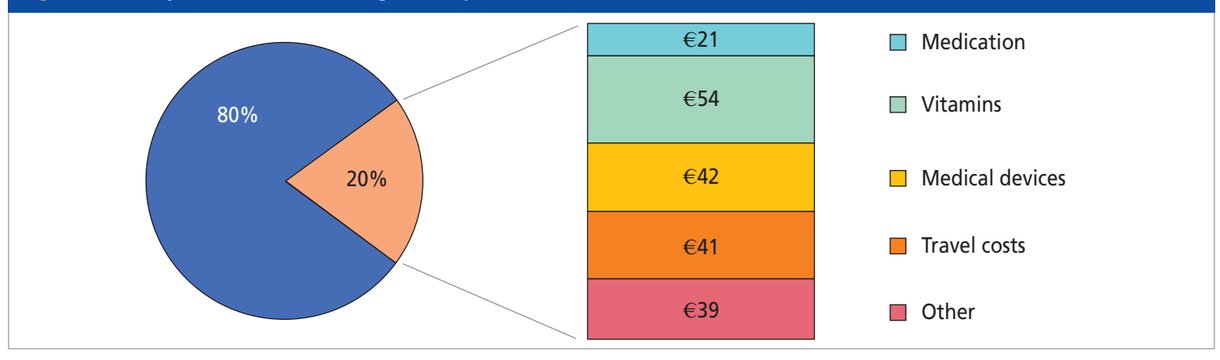
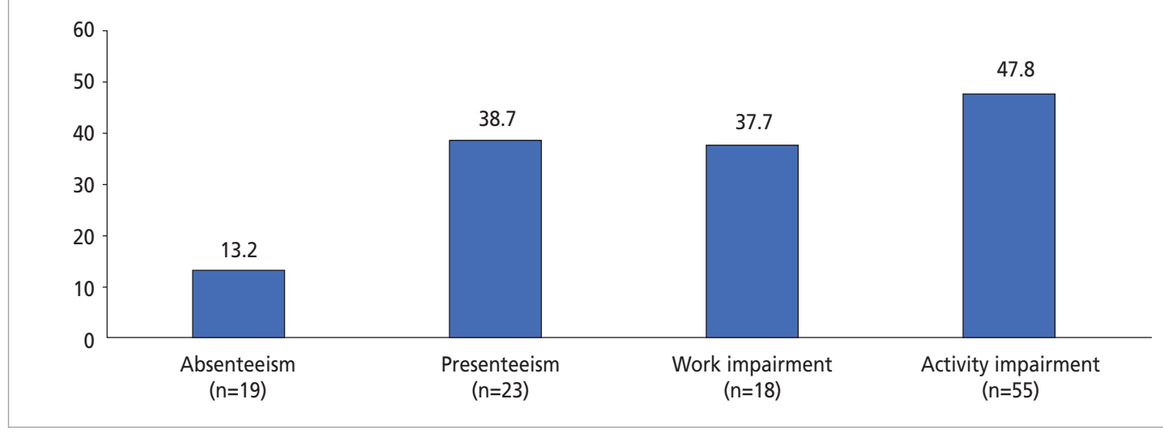


Figure 4. Work productivity and activity impairment (WPAI)



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