Module 5
What is the impact of Duchenne muscular dystrophy (DMD)?

Dyne | Medical Central

Module summary

Individuals with DMD can have substantially diminished quality of life[1]

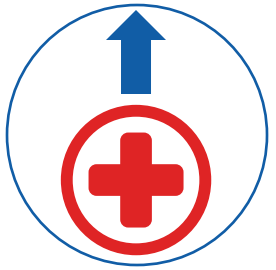
Physical, social, and emotional health is impacted[1]

DMD can impact education and social activities[2]

Many individuals attend a special school, and do not participate in organized leisure activities[2]

The burden of DMD extends to caregivers[3]

Some caregivers reduce their working hours or stop working completely due to their caregiving responsibilities[3]



DMD is associated with significant economic impact[3,4]

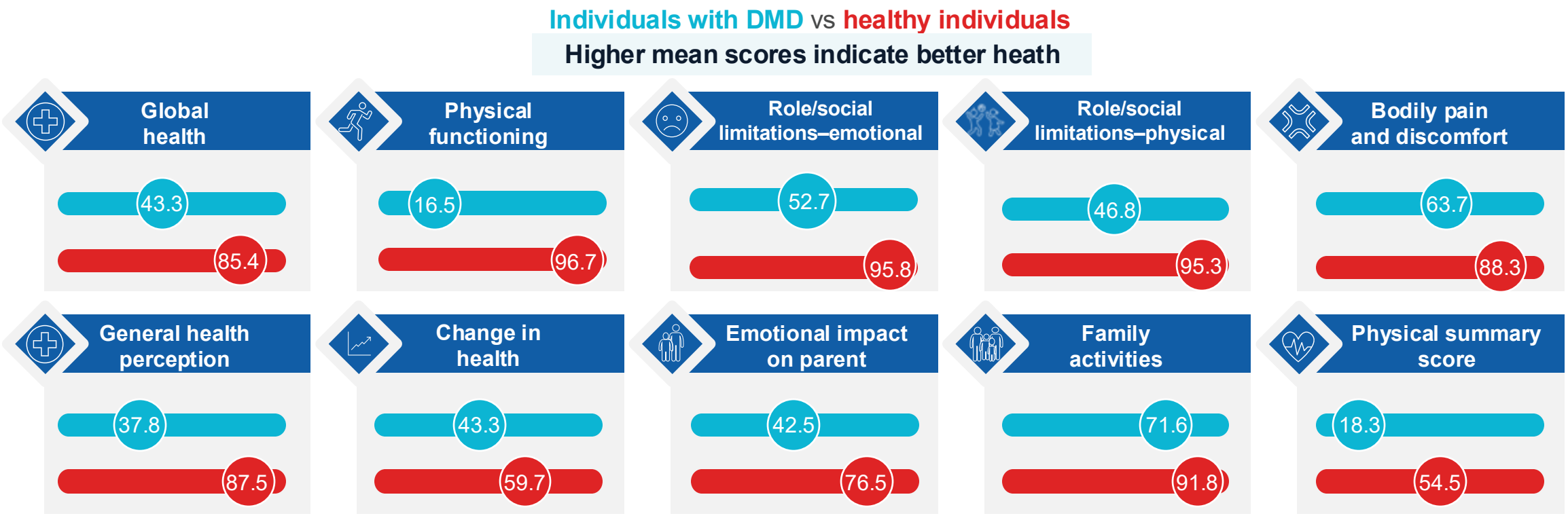
Healthcare resource utilization is substantially higher for individuals with DMD compared to controls[4]

DMD, Duchenne muscular dystrophy.

1. Baiardini I, et al. *J Child Neurol.* 2011;26:707–713; 2. Henzi BC, et al. *Eur J Paediatr Neurol.* 2025;56:107–114; 3. Landfeldt E, et al. *Dev Med Child Neurol.* 2018;60:987–996; 4. Klimchak AC, et al. *J Manag Care Spc Pharm.* 2021;27:1426–1437.

How does DMD impact patient quality of life?

In a cross-sectional survey of 27 individuals with DMD (mean [SD] age, 11.3 [5.1] years) and 27 caregivers evaluating burden of disease in Italy via the CHQ–Parent Form 50 and FSQ compared with heathy subjects (n=788; CHQ only):



Individuals with DMD showed poorer scores vs healthy individuals for physical, social, and emotional health

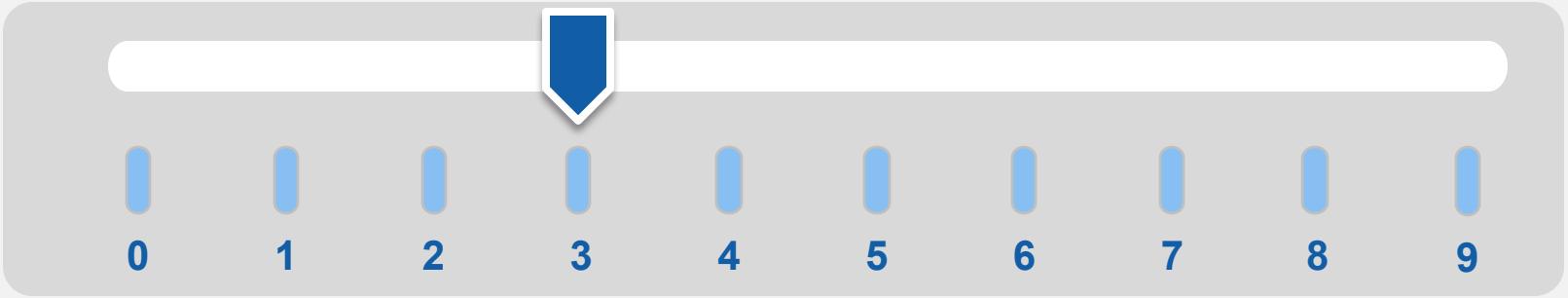
CHQ, Child Health Questionnaire; DMD, Duchenne muscular dystrophy; FSQ, Family Strain Questionnaire; SD, standard deviation.

Baiardini I, et al. *J Child Neurol.* 2011;26:707–713.

What is the impact of DMD on individuals' education and social activities?

In a cross-sectional survey* assessing school problems, social participation, and executive functions of 40 males with DMD aged 8–18 years old from the Swiss Registry for Neuromuscular Disorders:

Median sum score for school problems



Score of 0 indicates no difficulties

9 indicates more difficulties as special support needed, change of school, reduced concentration

53% attended a special school

18% participated in sports activities

58% did not attend any organized leisure activity

*Survey questionnaire included 57 questions that were specifically designed for this study to assess mobility, school problems, and social participation. The answers to each question were dichotomized with the value 1 denoting more difficulties or more advanced disease and 0 denoting less difficulties. Single questions of the questionnaire were grouped into three major domains: mobility, school problems, and social participation. Out of the dichotomized answers sum scores were calculated for each domain separately. The domain 'school problems' assessed a participant's performance at school, such as school support, concentration, and need to change school with 9 items and a total sum score of 0 to 9.

DMD, Duchenne muscular dystrophy.

Henzi BC, et al. *Eur J Paediatr Neurol.* 2025;56:107–114.

Does the burden of DMD extend to caregivers?

Systematic review of 21 articles reporting on caregiver burden of DMD:[1]

Estimated overall caregiver burden via mean ZBI score:

0 (low burden) — 88* (high burden)

EU† + US	29
EU‡	28
Brazil	26

Estimated impact on caregiver HRQoL via mean EQ-5D-3L score:

0 (death) — 1 (perfect health)

EU‡	0.71
EU† + US	0.81
Netherlands	0.87

Estimated proportion of caregivers that reduced working hours or stopped working completely to care for patients with DMD:

UK	49%
Germany	43%
Italy	29%
US	27%

*22 items rated on a 5-point Likert scale that ranges from 0 (never) to 4 (nearly always) with the sum scores ranging between 0 and 88. A score of 17 or more is considered high burden.[2]
†Germany, Italy, and the UK; ‡Bulgaria, France, Germany, Hungary, Italy, Spain, Sweden, and the UK.[1]
DMD, Duchenne muscular dystrophy; EU, European Union; EQ-5D, EuroQol-5D; HRQoL, health-related quality of life; US, United States; ZBI, Zarit Burden Interview.
1. Landfeldt E, et al. *Dev Med Child Neurol.* 2018;60:987–996; 2. Al-Rawashdeh SY, et al. *J Cardiovasc Nurs.* 2016;31:E21–E28.

How does DMD impact healthcare resource utilization and health expenditure?

Monthly healthcare resource use for individuals with DMD, 1,964 with commercial coverage and 2,007 with Medicaid, over a median (IQR) follow-up of 1.7 (0.9–3.4) and 3.1 (1.6–4.7) years, respectively[1]

Type of resource	Commercial		Medicaid	
	DMD	Comparison cohort*	DMD	Comparison cohort*
	n=1,420	n=4,486	n=1,722	n=5,395
Distinct medications dispensed	↑ 2.4 (1.5–4.4)	1.3 (1.0–2.0)	↑ 3.5 (1.9–6.6)	1.5 (0.0–2.6)
Any hospitalization, n (%)	↑ 368 (25.9)	162 (3.6)	↑ 535 (31.1)	345 (6.4)
Length of stay per hospitalization, days	↑ 4.0 (2.0–7.0)	3.0 (2.0–5.0)	↑ 4.3 (2.0–8.2)	3.4 (2.0–6.0)
Any ED visit, n (%)	↑ 679 (47.8)	1,215 (27.1)	↑ 1,113 (64.6)	2,625 (48.7)
Outpatient visits	↑ 1.4 (0.7–3.1)	0.2 (0.1–0.5)	↑ 3.8 (1.3–13.9)	0.3 (0.1–0.7)
≥1 GP visit, n (%)	↑ 916 (64.5)	2,074 (46.2)	↑ 1,179 (68.5)	2,670 (49.5)
≥1 specialist visit n (%)	↑ 1,378 (97.0)	3,509 (78.2)	↑ 1,293 (75.1)	3,441 (63.8)

Data are median (IQR) unless otherwise specified

Estimated mean per-patient annual direct cost of DMD[2†]

7× higher than the mean per-capita health expenditure

DMD is associated with higher healthcare resource utilization compared with controls, as well as significant economic impact[1,2]

*The comparison cohorts of each insurance type were drawn from all individuals without a diagnostic code for muscular dystrophy over the study period and matched to DMD on age at index date, sex, and calendar month of the index date.
†Local reference prices were applied to healthcare resource data to produce estimates of direct cost of illness and were presented in 2012 international US dollars, calculated using purchasing power parity data from Eurostat.
DMD, Duchenne muscular dystrophy; ED, emergency department; GP, general practitioner; IQR, interquartile range.
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1. Klimchak AC, et al. *J Manag Care Spc Pharm*. 2021;27:1426–1437; 2. Landfeldt E, et al. *Neurology*. 2014;83:529–536.