



Module 5

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



Module summary



Individuals with DMD can have substantially diminished quality of life¹

Physical, social, and emotional health is impacted¹



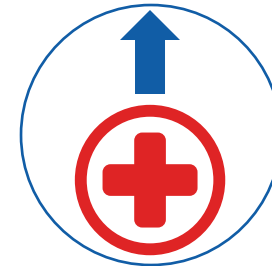
DMD can impact education and social activities²

Many individuals attend a special school, and do not participate in organized leisure activities²



The burden of DMD extends to caregivers³

Some caregivers reduce their working hours or stop working completely due to their caregiving responsibilities³



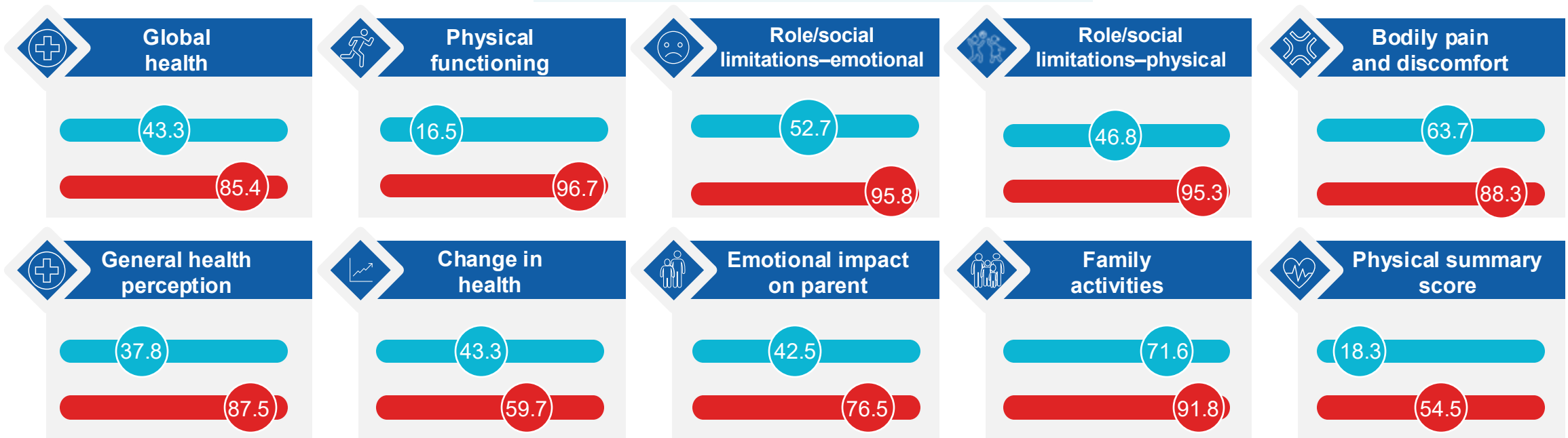
DMD is associated with significant economic impact^{3,4}

Healthcare resource utilization is substantially higher for individuals with DMD compared to controls⁴

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 healthy subjects (n=788; CHQ only):

Individuals with DMD vs healthy individuals
Higher mean scores indicate better health



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

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**

0

1

2

3

4

5

6

7

8

9

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.

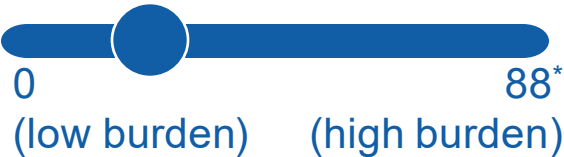
Henzi BC, et al. *Eur J Paediatr Neurol*. 2025;56:107–114.

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:



		EU [†] + US	29
		EU [†]	28
		Brazil	26



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



		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, respectively¹

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

*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.

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}